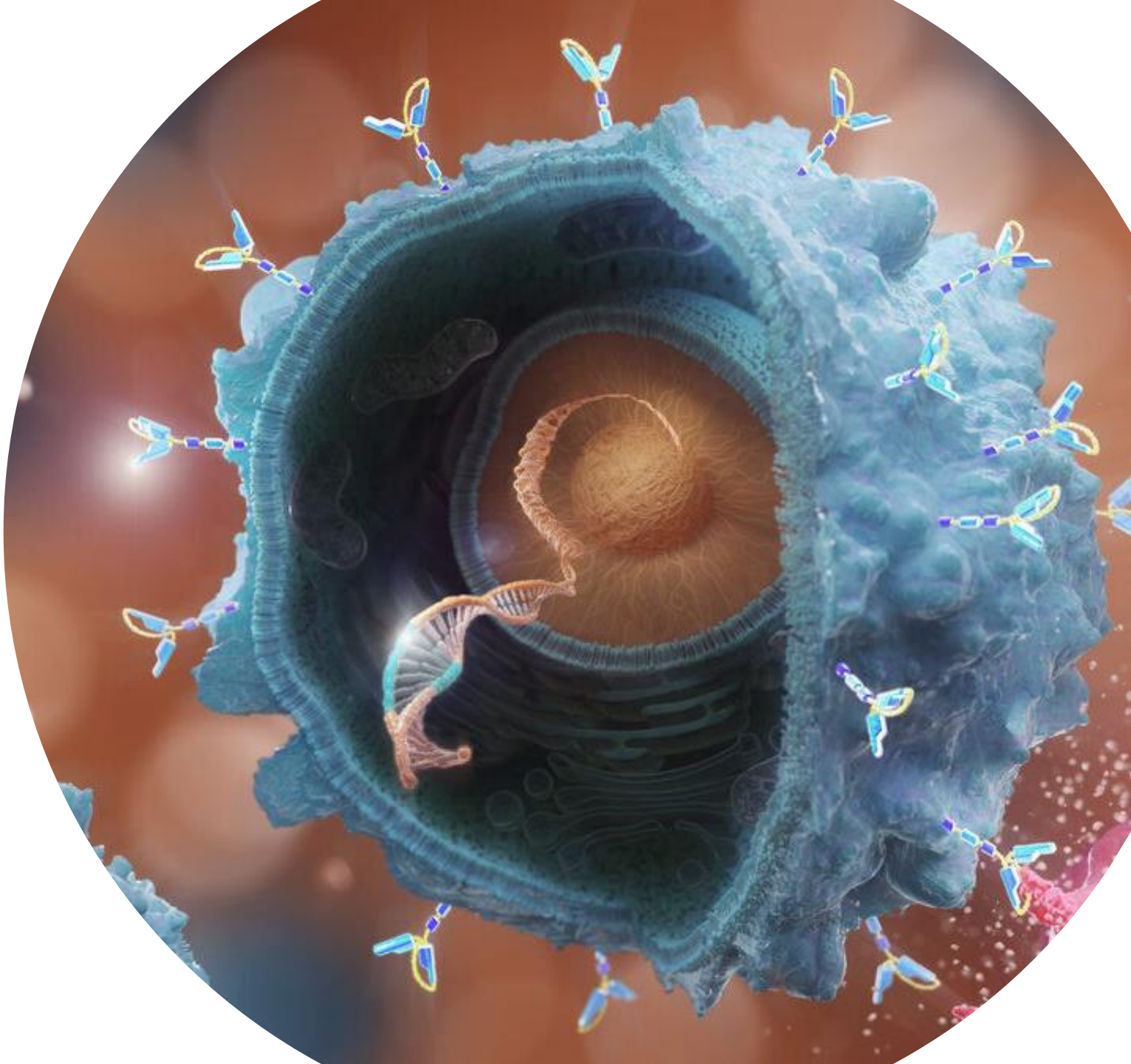
AstraZeneca

# Clinical Trials Appendix

## FY 2024 Results Update

6 February 2025



# Pipeline
# at a glance

Across five focus therapy areas:

**Oncology**

**BioPharmaceuticals**

CVRM | R&I | V&I

**Rare Disease**

**191**

projects in our development pipeline

**19**

new molecular entities (NME) in our late-stage pipeline

**130**

new molecular entities (NME) or major lifecycle management (LCM) projects in Phase II and Phase III

**31**

regulatory approvals in major markets since FY 2023



As of 6 February 2025.
Appendix: Glossary.

# Key upcoming pipeline catalysts: 2025 and 2026

Oncology BioPharmaceuticals Rare Disease

| | H1 2025 | H2 2025 | 2026 |
|---|---|---|---|
| **Regulatory decision[1,2]** | ***Tagrisso*** – *EGFR*m NSCLC (unresectable, Stg. III) (LAURA) (JP)<br>***Calquence*** – CLL (1L, treat-to-progression) (ELEVATE-TN) (CN)<br>***Imfinzi*** – early-stage NSCLC (perioperative) (AEGEAN) (EU, CN)<br>***Imfinzi*** – limited-stage SCLC (ADRIATIC) (JP)<br>***Imfinzi*** – muscle-invasive bladder cancer (NIAGARA)<br>***Enhertu*** – HER2-low and -ultralow met. breast cancer (DESTINY-Breast06) (EU)<br>***Datroway*** – HR+ HER2- met. breast cancer (2L+) (TROPION-Breast01) (EU)<br>***Wainua*** – ATTRv-PN (NEURO-TTRansform) (EU)<br>***Ultomiris*** – gMG (CHAMPION-MG) (CN)<br>**acoramidis** – ATTR-CM ( ALXN2060-TAC-302) (JP) | ***Calquence*** – MCL (1L) (ECHO) (EU, JP)<br>***Calquence*** – CLL (1L fixed duration) (AMPLIFY)<br>***Imfinzi*** – early-stage NSCLC (perioperative) (AEGEAN) (JP)<br>***Imfinzi*** – limited-stage SCLC (ADRIATIC) (EU, CN)<br>***Enhertu*** – HER2-low and ultralow met. breast cancer (DESTINY-Breast06) (JP)<br>***Truqap*** – HR+ HER2- met. breast cancer (2L) (CAPItello-291) (CN)<br>***Datroway*** – HR + HER2- breast cancer (2L+) (TROPION-Breast01) (CN)<br>***Datroway*** – *EGFR*m NSCLC (later line) (TROPION-Lung05)<br>***Ultomiris*** – NMOSD (CHAMPION-NMOSD) (CN)<br>***Koselugo*** – adult NF1-PN (KOMET) | ***Imfinzi + Imjudo*** – NSCLC (1L) (POSEIDON) (CN)<br>***Tezspire*** – severe asthma (DIRECTION) (CN)<br>***Wainua*** – ATTRv-PN (NEURO-TTRansform) (CN) |
| **Key Phase III data readouts** | ***Enhertu*** – high-risk HER2+ early breast cancer (neoadj.) (DESTINY-Breast11)<br>***Datroway*** – met. TNBC (TROPION-Breast02)<br>***Breztri*** – severe asthma (KALOS/LOGOS)<br>**eneboparatide** – hypoparathyroidism (CALYPSO) | ***Tagrisso + Orpathys*** – *EGFR*m NSCLC (SAFFRON)<br>***Imfinzi*** – resectable GC/GEJC (MATTERHORN)<br>***Imfinzi*** – non-muscle-invasive bladder cancer (POTOMAC)<br>***Imfinzi*** – muscle-invasive bladder cancer (VOLGA)<br>***Enhertu*** – high-risk early HER2+ breast cancer (adjuvant) (DESTINY-Breast05)<br>***Enhertu*** – HER2+ met. breast cancer (1L) (DESTINY-Breast09)<br>***Datroway + Imfinzi*** – Non-squamous/Non-squamousTROP2+ NSCLC (1L) (AVANZAR)<br>**camizestrant** – *ESR1*m HR+ HER2- met. breast cancer (1L switch) (SERENA-6)<br>**ceralasertib** – post-IO NSCLC (LATIFY)<br>***Fasenra*** – moderate to severe COPD (RESOLUTE)<br>***Saphnelo*** – moderate to severe SLE (TULIP-SC)<br>**baxdrostat** – uncontrolled hypertension (BaxHTN)<br>***Ultomiris*** – HSCT-TMA (ALXN1210-TM-313/-314)<br>**anselamimab** – AL amyloidosis (Mayo Stg. IIIa/b) (CAEL101-302)/ CAEL101-301)<br>**gefurulimab** – myasthenia gravis (ALXN1720-MG-301)<br>**efzimfotase alfa** – hypophosphatasia (HICKORY/CHESTNUT) | ***Imfinzi*** – early HCC (EMERALD-2)<br>***Imfinzi*** – locoregional HCC (EMERALD-3)<br>***Truqap*** – mCRPC (CAPItello-280)<br>***Datroway*** – NSQ NSCLC (1L) (TROPION-Lung07)<br>***Datroway + Tagrisso*** – *EGFR*m NSCLC (2L) (TROPION-Lung15)<br>***Datroway*** – PD-L1+ met. TNBC (1L) (TROPION-Breast05)<br>**camizestrant** – HR+ HER2- met. breast cancer (1L) (SERENA-4)<br>**AZD0901** – CLDN18.2+ gastric cancer (2L+) (CLARITY-Gastric01)<br>***Saphnelo*** – lupus nephritis (IRIS)<br>***Saphnelo*** – systemic sclerosis (DAISY)<br>***Wainua*** – ATTR-CM (CARDIO-TTRansform)<br>**tozorakimab** – COPD (OBERON/TITANIA)<br>**tozorakimab** – COPD (MIRANDA)<br>**tozorakimab** – LRTD (TILIA)<br>**efzimfotase alfa** – hypophosphatasia (MULBERRY) |

Key upcoming pipeline catalysts are defined by a threshold of non-risk adjusted global peak year revenue expectations as of 6 February 2025

[1]Regulatory decision includes programmes under review in a major market

[2]Inclusion dependent on status of regulatory submission and/or submission acceptance in regions in which submission acceptance is granted

As of 6 February 2025.

Appendix: Glossary.

# Clinical Trials Appendix: selected highlights

| | BioPharmaceuticals | Oncology | Rare Disease |
|---|---|---|---|
| **Approved medicines: key LCM** | AIRSUPRA™ (albuterol 90 mcg/budesonide 80 mcg) Inhalation Aerosol<br>Saphnelo™ (anifrolumab-fnia) Intravenous Use 300 mg/vial<br>BREZTRI AEROSPHERE™<br>TEZSPIRE™ (tezepelumab-ekko) Subcutaneous Injection 210 mg<br>Fasenra® (benralizumab) Subcutaneous Injection 30 mg<br>WAINUA™ (eplontersen) | TAGRISSO® osimertinib<br>ENHERTU®<br>DATROWAY® datopotamab deruxtecan-dlnk<br>CALQUENCE® (acalabrutinib) 100 mg capsules<br>Lynparza™ olaparib<br>IMFINZI® durvalumab Injection for Intravenous Use 50 mg/mL<br>IMJUDO® tremelimumab-actl Injection for Intravenous Use 20 mg/mL<br>Truqap™ capivasertib 160 mg • 200 mg tablets | ULTOMIRIS® (ravulizumab) injection for intravenous use<br>Koselugo® (selumetinib) 10 mg & 25 mg capsules |
| **Next-wave pipeline: registrational studies ongoing** | balcinrenone/dapagliflozin (MRM/SGLT2)<br>baxdrostat (aldosterone synthase inhibitor)<br>baxdrostat/dapagliflozin (ASI/SGLT2)<br>zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2)<br>tozorakimab (IL-33 ligand mAb) | camizestrant (next generation oral SERD)<br>saruparib (PARP1 inhibitor)<br>rilvegostomig (PD-1/TIGIT bispecific)<br>volrustomig (PD-1/CTLA-4 bispecific)<br>AZD0901 (CLDN18.2 ADC)<br>AZD0486 (CD19/CD3 TCE) | ALXN2220 (TTR depleter)<br>efzimfotase alfa (enzyme replacement therapy)<br>eneboparatide (PTH 1 agonist)<br>gefurulimab (C5 inhibitor) |



As of 06 February 2025.
Appendix: Glossary.

# Project movements since Q3 2024 update

## New to Phase I

**NME**

**AZD7760**

anti-staph aureus antibody combination targeting AT And ClfA prevention of staph bloodstream infections in haemodialysis patients

**Additional indication**

**AZD7003 (China)**

GPC3 CAR-T squamous non-small cell lung cancer

## New to Phase II

**NME**

**AZD2389**

anti-fibrotic mechanism metabolic dysfunction-associated steatohepatitis

**Additional indication**

**AZD0486 - SOUNDTRACK-B**

CD19/CD3 next-generation bispecific T-cell engager B-cell non-Hodgkin lymphoma

**Life-cycle management**

***Enhertu* DESTINY-PanTumor03#**

HER2 targeting ADC HER2 expressing solid tumours

## New to pivotal trial

**Additional indication**

**rilvegostomig ARTEMIDE-Lung02#**

PD-1/TIGIT bispecific mAb squamous NSCLC 1L

## New to registration

**Life-cycle management**

***Calquence* + venetoclax + obinutuzumab AMPLIFY**

BTK inhibitor + BCL-2 inhibitor + anti-CD20 mAb 1st-line chronic lymphocytic leukaemia

***Datroway* (datopotamab deruxtecan) TROPION-Lung05#**

TROP2 ADC advanced or metastatic *EGFR*m NSCLC progressed on prior systemic therapies, including TKIs and platinum-based chemotherapy

***Koselugo* KOMET#**

MEK inhibitor neurofibromatosis type 1 adult

Phase progressions based on first subject in achievement
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

# Project movements since Q3 2024 update

## Removed from Phase I

**NME**

**ALXN1910**
next generation TNSALP ERT bone metabolism

## Removed from Phase II

**NME**

**AZD0171 + *Imfinzi* + CTx**
anti-LIF mAb + PD-L1 mAb + CTx 1st-line metastatic pancreatic ductal adenocarcinoma

**AZD4041#**
orexin 1 receptor antagonist opioid use disorder

**sabestomig**
PD-1/TIM3 bispecific mAb solid tumours

**vemircopan**
oral factor D inhibitor immunoglobulin A nephropathy/proliferative lupus nephritis

**mitiperstat**
myeloperoxidase COPD/heart failure with a preserved ejection fraction/NASH

## Removed from Phase III

## Approved/removed from registration

**NME**

***Datroway* (datopotamab deruxtecan) TROPION-Breast01#**
TROP2 ADC 2-3L HR+ HER2- breast cancer

***Kavigale* (sipavibart) SUPERNOVA**
SARS-CoV-2 LAAB prevention of COVID-19

**Life-cycle management**

***Enhertu* DESTINY-Breast06#**
HER2 targeting ADC post-ET HER2-low and -ultralow/HR+ breast cancer 2L

***Imfinzi* +/- *Imjudo* + CRT ADRIATIC#**
PD-L1 mAb +/- CTLA-4 mAb + CRT 1st-line limited-stage SCLC

Phase progressions based on first subject in achievement
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

# Q4 2024 Oncology new molecular entity[1] pipeline

## Phase I

21 New Molecular Entities

- AZD0022
  KRas G12D inhibitor solid tumours
- AZD0486
  CD19/CD3 TCE r/r B-cell non-Hodgkin lymphoma
- AZD0305
  GPRC5D ADC relapsed/refractory multiple myeloma
- AZD1390
  ATM inhibitor glioblastoma
- AZD3470
  PRMT5 inhibitor classic Hodgkin lymphoma, solid tumours
- AZD5851
  GPC3 CAR-T hepatocellular carcinoma
- AZD6422
  CLDN18.2 CAR-T solid tumours
- AZD8421
  CDK2 inhibitor solid tumours
- AZD9829
  CD123 TOP1i ADC AML, MDS
- NT-125#
  autologous, fully-individualized, multi-specific TCR therapy targeting neoantigens solid tumours
- volrustomig + lenvatinib
  PD-1/CTLA-4+VEGF advanced RCC
- AZD0486
  CD19/CD3 TCE B-cell acute lymphoblastic leukaemia
- AZD0120
  autologous anti-CD19 and anti-BCMA CAR-T cell immunotherapy multiple myeloma
- AZD0754
  STEAP2 CAR-T prostate cancer
- AZD2068
  EGFR cMET radioconjugate solid tumours
- AZD5492
  CD20 TITAN T-cell engager haematology
- AZD5863
  CLDN18.2 x CD3 bispecific antibody (HBM7022) solid tumours
- AZD7003 (China)
  GPC3 CAR-T hepatocellular carcinoma/ squamous non-small cell lung cancer
- AZD9592
  EGFR/cMET TOP1i ADC solid tumours
- NT-112#
  TGFBR2 KO armored TCR-T targeting KRAS G12D solid tumour
- NT-175#
  TGFBR2 KO armored TCR-T targeting TP53 R175H solid tumours

## Phase II

14 New Molecular Entities

- AZD0486 SOUNDTRACK-B
  CD19/CD3 next-generation bispecific T-cell engager B-cell non-Hodgkin lymphoma
- AZD0901
  CLDN18.2 MMAE ADC solid tumours
- AZD5335
  anti-FRα TOP1i ADC ovarian cancer, lung adenocarcinoma
- puxitatug samrotecan (AZD8205)
  B7-H4 targeting ADC solid tumours
- AZD9574
  PARP inhibitor advanced solid malignancies
- camizestrant
  SERD HR+ breast cancer
- ceralasertib
  ATR inhibitor solid tumours
- FPI-2265#
  PSMA radioconjugate prostate cancer
- IPH5201 + *Imfinzi*#
  CD39 + PD-L1 neoadjuvant/adjuvant NSCLC
- rilvegostomig ARTEMIDE-01#
  PD-1/TIGIT bispecific mAb solid tumours
- saruparib
  PARP1Sel solid tumours
- volrustomig
  PD-1/CTLA-4 solid tumours
- volrustomig eVOLVE-01
  PD-1/CTLA-4 bispecific mAb NSCLC
- volrustomig eVOLVE-02
  PD-1/CTLA-4 bispecific mAb cervical cancer, head and neck squamous cell carcinoma

## Phase III

17 New Molecular Entities

- AZD0486 SOUNDTRACK-F1
  CD19/CD3 TCE follicular lymphoma
- AZD0901 CLARITY-Gastric01
  CLDN18.2 MMAE ADC gastric 2L+
- camizestrant + palbociclib SERENA-4
  SERD+CDK4/6i 1L HR+ HER2- breast cancer
- camizestrant ± abemaciclib CAMBRIA-2
  SERD +/- CDK4/6i ER+/HER2- early breast cancer
- *Imfinzi* +/- oleclumab +/- monalizumab PACIFIC-9#
  PD-L1+NKG2A or PD-L1+CD73 unresectable stage III NSCLC
- rilvegostomig ARTEMIDE-Lung03#
  PD-1/TIGIT bispecific mAb non-squamous NSCLC 1L
- saruparib EvoPAR-Breast01
  PARP1Sel BRCA/PALB2m HR+ve metastatic breast cancer
- volrustomig eVOLVE-HNSCC
  PD-1/CTLA-4 bispecific mAb unresected locally advanced HNSCC
- volrustomig eVOLVE-Meso
  PD-1/CTLA-4 bispecific mAb 1L unresectable malignant pleural mesothelioma
- camizestrant + CDK4/6i SERENA-6
  SERD+CDK4/6i 1L HR+ HER2- ESR1m breast cancer
- camizestrant CAMBRIA-1
  SERD HR+ HER2- extended adjuvant breast cancer
- ceralasertib + *Imfinzi* LATIFY
  ATR inhibitor + PDL-1 NSCLC
- rilvegostomig ARTEMIDE-Lung02#
  PD-1/TIGIT bispecific mAb squamous NSCLC 1L
- rilvegostomig ARTEMIDE-Biliary01#
  PD-1/TIGIT bispecific mAb adjuvant biliary tract cancer
- saruparib EvoPAR-Prostate01
  PARP1Sel metastatic castration-sensitive prostate cancer
- volrustomig eVOLVE-Cervical
  PD-1/CTLA-4 bispecific mAb locally advanced cervical cancer
- volrustomig eVOLVE-Lung02
  PD-1/CTLA-4 bispecific mAb 1L metastatic NSCLC

## Under review

0 New Molecular Entities

Phase progressions based on first subject in achievement
1. Includes additional indications for assets where the lead is not yet launched
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

● Precision medicine approach being explored

# Q4 2024 Oncology lifecycle management[1] pipeline

## Phase I
0 Projects

## Phase II
9 Projects

- *Enhertu* (platform) DESTINY-Breast07# HER2 targeting ADC HER2+ breast cancer
- *Enhertu* DESTINY-PanTumor03# HER2 targeting ADC HER2 expressing solid tumours
- *Enhertu* DESTINY-PanTumour01# HER2 ADC HER2 mutant tumours
- *Imfinzi* (platform) BEGONIA PD-L1 1L metastatic TNBC
- *Imfinzi* (platform) HUDSON PD-L1+multiple novel ONC therapies post IO NSCLC
- *Imfinzi* (platform) NeoCOAST-2# PD-L1 mAb + multiple novel oncology therapies NSCLC
- *Tagrisso* + *Orpathys* SAVANNAH# EGFR+MET advanced EGFRm NSCLC
- *Tagrisso* ORCHARD platform study# EGFR+multiple novel ONC therapies 2L EGFRm osimertinib-resistant NSCLC
- *Truqap* AKT prostate cancer

## Phase III
38 Projects

- *Calquence* + R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL
- *Datroway* + *Imfinzi* AVANZAR# TROP2 ADC + PD-L1 + CTx Non-squamous/Non-squamous TROP2 + NSCLC (1L)
- *Datroway* +/- *Imfinzi* TROPION-Breast03# TROP2 ADC +/- PD-L1 adjuvant residual disease TNBC
- *Datroway* TROPION-Lung08# TROP2 ADC 1L metastatic NSCLC
- *Enhertu* DESTINY-Breast11# HER2 ADC neoadjuvant HER2+ breast cancer
- *Enhertu* DESTINY-Gastric04# HER2 ADC HER2+ gastric 2L
- *Imfinzi* + CRT KUNLUN PD-L1+CRT locally-advanced ESCC
- *Imfinzi* + FLOT MATTERHORN# PD-L1+CTx neoadjuvant/adjuvant gastric cancer
- *Imfinzi* + *Imjudo* + TACE +/- lenvatinib EMERALD-3 PD-L1+CTLA4+VEGF+/-chemoembolisation locoregional HCC
- *Imfinzi* POTOMAC PD-L1 non-muscle invasive bladder cancer
- *Lynparza* MONO-OLA1# PARP 1L BRCAwt ovarian cancer
- *Tagrisso* ADAURA2 EGFR adjuvant EGFRm NSCLC stage Ia2-Ia3 following complete tumour resection
- *Truqap* + docetaxel CAPItello-280 AKT+docetaxel mCRPC prostate cancer
- *Datroway* + rilvegostomig TROPION-Lung12 # TROP2 ADC + PD-1/TIGIT Stage I adenocarcinoma NSCLC who are ctDNA-positive or have high-risk pathological features
- *Datroway* + *Tagrisso* TROPION-Lung15# TROP2 ADC + EGFR inhibitor 2L advanced or metastatic EGFRm NSCLC
- *Datroway* + *Imfinzi* TROPION-Breast04# TROP2 ADC + PD-L1 perioperative triple negative or HR-low/HER2-negative breast cancer
- *Datroway* + pembrolizumab TROPION-Lung07# TROP2 ADC 1L NSCLC PD-L1 <50% non-squamous
- *Enhertu* + rilvegostomig DESTINY-BTC01 HER2 targeting ADC + PD-1/TIGIT bispecific mAb 1L HER2+ biliary tract cancer
- *Enhertu* DESTINY-Breast09# HER2 ADC HER2+ breast cancer 1L
- *Imfinzi* + CRT PACIFIC-5 (China)# PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi* + EV +/- *Imjudo* VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA-4 MIBC
- *Imfinzi* + VEGF + TACE EMERALD-1# PD-L1+VEGF+TACE locoregional HCC
- *Imfinzi* post-SBRT PACIFIC-4# PD-L1 mAb post-SBRT stage I/II NSCLC
- *Orpathys* + *Imfinzi* SAMETA# MET+PD-L1 1L papillary renal cell carcinoma
- *Tagrisso* +/- CTx neoadjuvant NeoADAURA EGFR+/-CTx stage II/III resectable EGFRm NSCLC
- *Truqap* + *Faslodex* + palbociclib CAPItello-292 AKT+fulvestrant+CDK4/6 1L triplet in early relapse/ET resistant locally advanced or mBC
- *Datroway* + rilvegostomig TROPION-Lung10# TROP2 ADC+PD-1/TIGIT locally advanced or metastatic non-squamous NSCLC with high PD-L1 expression (TC ≥50%) and without actionable genomic alterations
- *Datroway* TROPION-Breast02# TROP2 ADC 1L triple negative breast cancer
- *Datroway* + *Imfinzi* TROPION-Breast05# TROP2 ADC + PD-L1 1L triple negative breast cancer
- *Datroway* + *Tagrisso* TROPION-Lung14# TROP2 ADC + EGFR inhibitor 1L EGFRm NSCLC
- *Enhertu* DESTINY-Breast05# HER2 ADC HER2+ post-neoadjuvant high-risk breast cancer
- *Enhertu* DESTINY-Lung04# HER2 ADC HER2m NSCLC 1L
- *Imfinzi* + domvanalimab (AB154) PACIFIC-8# PD-L1+TIGIT+CTx unresectable stage III NSCLC
- *Imfinzi* + *Imjudo* + SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer
- *Imfinzi* + VEGF EMERALD-2# PD-L1+VEGF adjuvant HCC
- *Lynparza* + *Imfinzi* + bevacizumab DUO-O# PARP+PD-L1+VEGF 1L ovarian cancer
- *Tagrisso* + *Orpathys* SAFFRON# EGFR + MET advanced EGFRm non-small cell lung cancer
- *Truqap* + abiraterone CAPItello-281 AKT+abiraterone PTEN deficient mHSPC

## Under review
3 Projects

- *Calquence* + venetoclax + obinutuzumab AMPLIFY BTK+BCL-2+anti-CD20 1L CLL
- *Datroway* TROPION-Lung05# TROP2 ADC advanced or metastatic EGFRm NSCLC progressed on prior systemic therapies, including TKIs and platinum-based chemotherapy
- *Imfinzi* + CTx NIAGARA PD-L1+CTx muscle invasive bladder cancer

Phase progressions based on first subject in achievement
1. Includes significant lifecycle management projects and parallel indications for assets beyond Phase III
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

● Precision medicine approach being explored

# Q4 2024 BioPharmaceuticals new molecular entity[1] pipeline

## Phase I
15 New Molecular Entities

- AZD0120 autologous anti-CD19 and anti-BCMA CAR-T cell immunotherapy systemic lupus erythematosus
- AZD0292 pseudomonas Psl-PcrV bispecific mAb non-CF bronchiectasis
- AZD1705 lipid lowering cardiovascular disease
- AZD4144 inflammation modulator cardiorenal disease
- AZD6793 IRAK4 inhibitor inflammatory diseases
- AZD7760 mAb combination targeting S aureus virulence factors prevention of Staph aureus infection
- AZD9550 GLP-1R glucagon dual agonist non-alcoholic steatohepatitis
- mRNA VLP vaccine mRNA-VLP vaccine prevention of COVID-19
- AZD0233 CX3CR1 dilated cardiomyopathy
- AZD1163 bispecific antibody rheumatoid arthritis
- AZD2373 podocyte health nephropathy
- AZD5148 anti-clostridioides difficile TcdB mAb reduction of *C.diff* recurrence
- AZD6912 siRNA rheumatoid arthritis
- AZD8965 inhibition of arginase enzyme idiopathic pulmonary fibrosis
- MEDI1814# amyloid beta mAb Alzheimer's disease

## Phase II
17 New Molecular Entities

- atuliflapon FLAP asthma
- AZD2389 anti-fibrotic mechanism metabolic dysfunction-associated steatohepatitis
- AZD3427 relaxin mimetic heart failure
- AZD5004 oral GLP-1 receptor agonist T2D/chronic weight management
- AZD6234 peptide chronic weight management in overweight or obesity
- AZD8630# inhaled TSLP FAb asthma
- IVX-A12 virus-like particle (VLP) vaccine RSV and human metapneumovirus (hMPV)
- MEDI7352 NGF/TNF OA pain / PDN
- zibotentan/dapagliflozin endothelin A receptor antagonist/SGLT2i liver cirrhosis
- AZD0780 PCSK9 dyslipidemia
- AZD2693 NASH resolution non-alcoholic steatohepatitis
- AZD4604 inhaled JAK1 inhibitor asthma
- AZD5462# RXFP1 agonist heart failure
- AZD7798 humanised monoclonal antibody targets T-cells subset Crohn's disease
- balcinrenone/dapagliflozin MR modulator + SGLT2 inhibitor CKD
- MEDI0618 PAR2 antagonist mAb migraine
- tozorakimab FRONTIER 3 IL-33 asthma

## Phase III
6 New Molecular Entities

- balcinrenone/dapagliflozin MR modulator + SGLT2 inhibitor heart failure with CKD
- baxdrostat BaxHTN aldosterone synthase inhibitor hypertension
- baxdrostat/dapagliflozin aldosterone synthase inhibitor and reversible inhibitor of SGLT2 CKD
- tozorakimab OBERON TITANIA PROSPERO MIRANDA IL-33 COPD
- tozorakimab TILIA IL-33 severe viral lower respiratory tract disease
- zibotentan/dapagliflozin endothelin A receptor antagonist/SGLT2i CKD with high proteinuria

## Under review
0 New Molecular Entities

Phase progressions based on first subject in achievement
1. Includes additional indications for assets where the lead is not yet launched
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

Precision medicine approach being explored

# Q4 2024 BioPharmaceuticals life cycle management[1] pipeline

## Phase I
0 Projects

## Phase II
1 Project

- *Tezspire* COURSE#
  TSLP chronic obstructive pulmonary disease

## Phase III
13 Projects

- *Breztri*/Trixeo (PT010) KALOS LOGOS
  LABA/LAMA/ICS asthma
- *Breztri*/Trixeo ATHLOS
  LABA/LAMA/ICS COPD cardiopulmonary exercise trial
- *Breztri*/Trixeo THARROS#
  LABA/LAMA/ICS cardiopulmonary outcomes trial in COPD
- *Fasenra* RESOLUTE#
  IL-5R chronic obstructive pulmonary disease
- *Fasenra* NATRON
  IL-5R hypereosinophilic syndrome
- *Saphnelo* DAISY#
  type I IFN receptor systemic sclerosis
- *Saphnelo* IRIS#
  type I IFN receptor mAb lupus nephritis
- *Saphnelo* JASMINE#
  type I IFN receptor mAb myositis
- *Saphnelo* LAVENDER#
  type I IFN receptor mAb cutaneous lupus erythematosus
- *Saphnelo* TULIP-SC#
  type I IFN receptor systemic lupus erythematosus (subcutaneous)
- *Tezspire* WAYPOINT#
  TSLP nasal polyps
- *Tezspire* CROSSING#
  TSLP eosinophilic oesophagitis
- *Wainua*#
  LICA ATTR-cardiomyopathy

## Under review
0 Projects

Phase progressions based on first subject in achievement
1. Includes significant lifecycle management projects and parallel indications for assets beyond Phase III
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

Precision medicine approach being explored

# Q4 2024 Rare Disease pipeline[1]

## Phase I
3 Projects

- ALXN1920
  kidney-targeted factor H fusion protein nephrology
- ALXN2030
  siRNA targeting complement C3 nephrology
- ALXN2080
  oral factor D healthy volunteers

## Phase II
2 Projects

- MEDI1341#
  alpha synuclein mAb multiple system atrophy/Parkinson's disease
- *Ultomiris*
  anti-complement C5 mAb proliferative lupus nephritis

## Phase III
8 Projects

- ALXN2220 DepleTTR-CM#
  TTR depleter transthyretin amyloid cardiomyopathy
- anselamimab
  fibril-reactive mAb amyloid light-chain amyloidosis
- efzimfotase alfa
  next generation TNSALP ERT hypophosphatasia
- eneboparatide CALYPSO
  parathyroid hormone receptor 1 hypoparathyroidism
- gefurulimab PREVAIL
  humanised bispecific VHH antibody generalised myasthenia gravis
- *Ultomiris*
  anti-complement C5 mAb haematopoietic stem cell transplant–associated thrombotic microangiopathy
- *Ultomiris* ARTEMIS
  anti-complement C5 mAb cardiac surgery-associated acute kidney injury
- *Ultomiris* I CAN
  anti-complement C5 mAb immunoglobulin A nephropathy

## Under review
2 Projects

- acoramidis#
  oral TTR stabiliser transthyretin amyloid cardiomyopathy
- *Koselugo* KOMET#
  MEK inhibitor neurofibromatosis type 1 adult

Phase progressions based on first subject in achievement
1. Includes new molecular entities and significant lifecycle management projects
# Partnered and/or in collaboration
As of 6 February 2025.
Appendix: Glossary.

Precision medicine approach being explored

# Designations in our pipeline

**4**
**Accelerated approvals**

- *Andexxa* acute major bleed (US)
- *Kavigale* SARS-CoV-2 LAAB prevention of COVID-19 (EU)
- *Calquence* r/r MCL ACE-LY-004 (US)
- *Enhertu* HER2 overexp tumors (DESTINY-PanTumor02) (US)

**10**
**Breakthrough / PRIME[1] / Sakigake[2]**

- *Tezspire* asthma NAVIGATOR (US)
- *Tezspire* COPD COURSE (US)
- tozorakimab severe viral LRTD TILIA (CN)
- *Calquence* r/r MCL ACE-LY-004 (US)
- *Calquence* CLL (1L) ELEVATE-TN (US)
- *Datroway* post-TKI NSCLC 3L+ TROPION-Lung05 (US)
- *Enhertu* HER2-overexpressing tumours DESTINY-PanTumor02 (US)
- *Enhertu* post-ET HER2low and -ultralow HR+ breast 1L DESTINY-Breast06 (US)
- *Imfinzi*+/-*Imjudo*+CRT LS-SCLC (1L) ADRIATIC (US)
- *Tagrisso* stage III EGFRm NSCLC LAURA (US)

**14**
**Fast Track**

- AZD0292 Psl-PcrV N3Y NCFBE (US)
- AZD3427 relaxin mimetic heart failure (US)
- AZD7760 Staph aureus mAbs-Hemodialysis (US)
- balci/dapa HF with CKD (US)
- *Saphnelo* SLE (US)
- tozorakimab COPD (US)
- tozorakimab severe viral LRTD (US)
- *Wainua* ATTR-Cardiomyopathy (US)
- camizestrant 1L HR+ HER2- *ESR1*m breast cancer SERENA-6 (US)
- *Orpathys* + *Tagrisso* NSCLC SAVANNAH/SAFFRON (US)
- *Truqap* + fulv HR+ breast (2L+) CAPItello-291 (US)
- ALXN2220 DepleTTR-CM (US)
- anselamimab AL amyloidosis CAEL101-301/2 (US)
- eneboparatide HypoPT (US)

**13**
**Priority Review**

- *Tezspire* asthma NAVIGATOR (US)
- *Calquence* MCL (1L) ECHO (US)
- *Datroway EGFR*m post-TKI NSCLC 3L+ TROPION-Lung05 (US)
- *Enhertu* HER2 overexpressing tumors DESTINY-PanTumor02 (US)
- *Enhertu* post-ET HER2low/ultralow HR+ breast 1L DESTINY-Breast06 (US)
- *Imfinzi* + CTx MIBC NIAGARA (US)
- *Imfinzi* + *Imjudo* HCC (1L) HIMALAYA (US)
- *Imfinzi* + *Imjudo* LS-SCLC ADRIATIC (US)
- *Lynparza* + abiraterone all-comers mCRPC (1L) PROpel (US)
- *Tagrisso* stage III *EGFR*m NSCLC LAURA (US)
- *Tagrisso* stage III *EGFR*m NSCLC LAURA (CN)
- *Truqap* + fulv HR+ breast (2L+) CAPItello-291 (US)
- ALNX2220 ATTR-CM (JP)

**24**
**Orphan**

- *Andexxa* acute major bleed (JP)
- *Fasenra* EGPA MANDARA (US)
- *Fasenra* HES NATRON (US)
- *Saphnelo* myositis JASMINE (US)
- *Saphnelo* systemic sclerosis (US)
- *Tezspire* EoE CROSSING (US)
- *Wainua* transthyretin-mediated amyloidosis (US)
- *Calquence* CLL (1L) ELEVATE-TN (US)
- *Calquence* CLL (1L) ELEVATE-TN (EU)
- Calquence r/r MCL ACE-LY-004 (US)
- *Imfinzi* + *Imjudo* LS-SCLC ADRIATIC (JP)
- *Imfinzi* +/- *Imjudo* HCC (1L) HIMALAYA (EU)
- *Imfinzi* +/- *Imjudo* HCC (1L) HIMALAYA (US)
- ALXN2220 ATTR-CM DepleTTR-CM (US)
- ALXN2220 ATTR-CM DepleTTR-CM (EU)
- ALXN2220 ATTR-CM DepleTTR-CM (JP)
- anselamimab AL amyloidosis CAEL101-301/2 (US)
- anselamimab AL amyloidosis CAEL101-301/2 (EU)
- gefurulimab myasthenia gravis PREVAIL (US)
- *Koselugo* NF1 adult 1L KOMET (US)
- *Koselugo* NF1 adult 1L KOMET (EU)
- *Koselugo* NF1 adult 1L KOMET (JP)
- *Koselugo* NF1 adult 1L KOMET (CN)
- *Ultomiris* HSCT-TMA ALXN1210-TM-313 (US)

**3**
**Qualified infectious disease product**

- AZD0292 Psl-PcrV N3Y NCFBE (US)
- AZD5148 C. difficile mAb - Prevention of Recurrence (US)
- AZD7760 prevention of Staph aureus infection (US)

ACCELERATED APPROVAL, these regulations allowed medicines for serious conditions that addressed an unmet medical need to be approved based on a surrogate endpoint

BREAKTHROUGH DESIGNATION is a process designed to expedite the development and review of medicines which may demonstrate substantial improvement over available therapy. [1]PRIME is a scheme launched by the EMA to enhance support for the development of medicines that target an unmet medical need. [2]SAKIGAKE is aimed at early introduction of innovative medicines, medical devices, etc. that are initially developed in Japan

FAST TRACK is a process designed to facilitate the development, and expedite the review of medicines to treat serious conditions and fill an unmet medical need

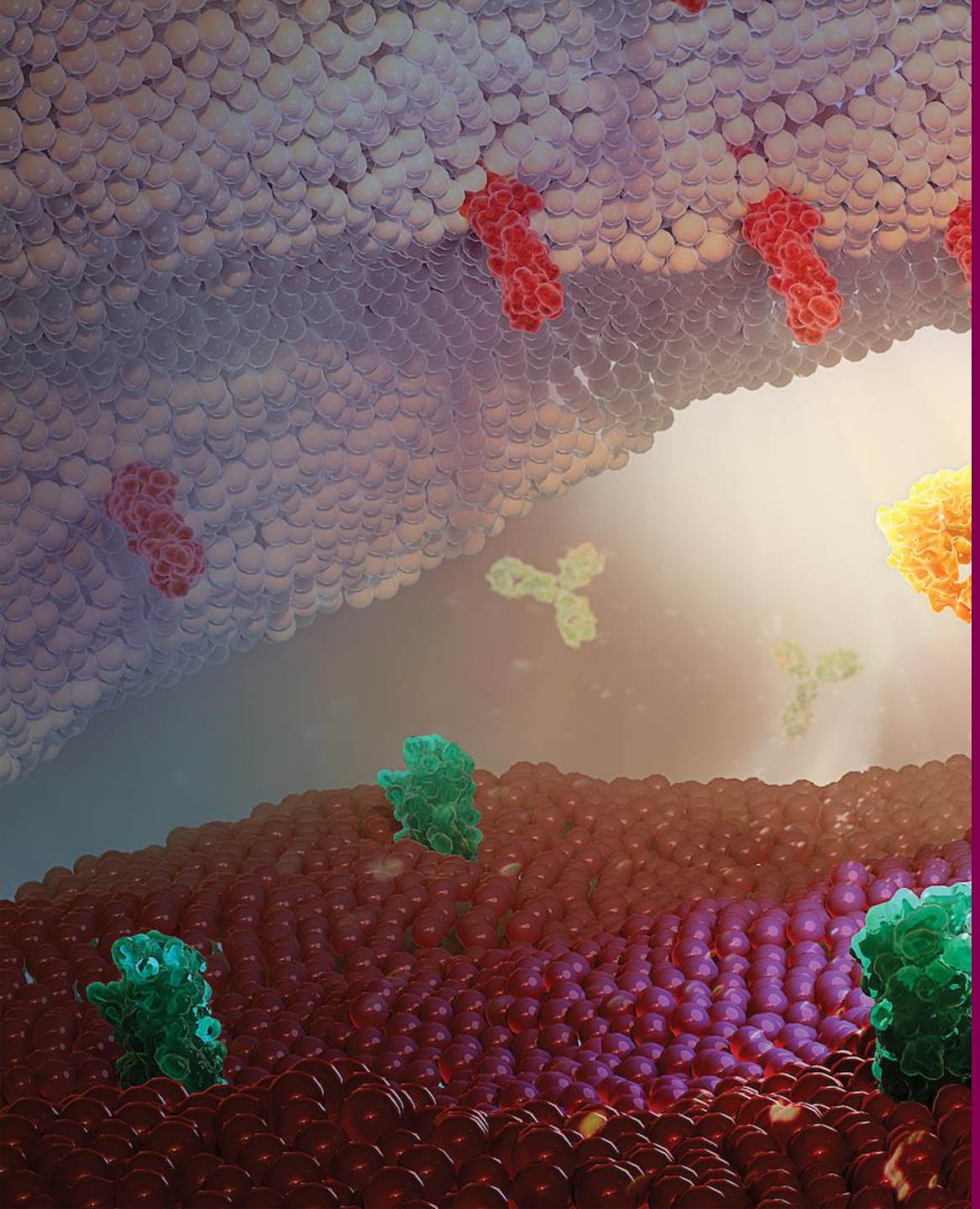
PRIORITY REVIEW DESIGNATION is the US FDA's goal to take action on an application within 6 months

ORPHAN DRUG DESIGNATION, intended for treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 patients in the US, or that affect more than 200,000 patients but are not expected to recover the costs of developing and marketing a treatment drug

QUALIFIED INFECTIOUS DISEASE PRODUCT designation confers particular advantages, including priority review by the US Food and Drug Administration (FDA) and fast-track designation, which can accelerate development of a product, as well as an additional five years' market exclusivity if a product is licensed.

Oncology BioPharmaceuticals Rare Disease

NOTE: excludes designations for projects which have launched in all applicable major markets
As of 6 February 2025.
Appendix: Glossary.



# Oncology:
## approved medicines and late-stage pipeline

AstraZeneca

# *Imfinzi* (PD-L1 mAb)

## Gastrointestinal cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>EMERALD-1<br>NCT03778957** | Locoregional HCC | 710 | • Arm 1: TACE in combination with *Imfinzi*<br>• Arm 2: TACE in combination with *Imfinzi* + bevacizumab<br>• Arm 3: TACE in combination with placebo | • Primary endpoint: PFS (Arm 2 vs. Arm 3)<br>• Secondary endpoints: PFS (Arm 1 vs. Arm 3) and OS | • FPCD: Q1 2019<br>• LPCD: Q3 2021<br>• Data readout: Q4 2023<br>• Primary endpoint met |
| **Phase III<br>EMERALD-2<br>NCT03847428** | HCC (adjuvant) | 908 | • Arm 1: *Imfinzi* + bevacizumab<br>• Arm 2: *Imfinzi* + placebo<br>• Arm 3: placebo + placebo | • Primary endpoint: RFS (Arm 1 vs. Arm 3)<br>• Secondary endpoints: RFS (Arm 2 vs. Arm 3), OS and RFS at 24 months | • FPCD: Q2 2019<br>• LPCD: Q2 2022<br>• Data anticipated: 2026 |
| **Phase III<br>KUNLUN<br>NCT04550260** | Locally advanced, unresectable ESCC | 640 | • Arm 1: *Imfinzi* + definitive CRT<br>• Arm 2: placebo + definitive CRT | • Primary endpoint: PFS<br>• Secondary endpoint: OS | • FPCD: Q4 2020<br>• LPCD: Q3 2023<br>• Data anticipated: 2026 |
| **Phase III<br>MATTERHORN<br>NCT04592913** | Resectable GC/GEJC | 900 | • Arm 1: *Imfinzi* + FLOT<br>• Arm 2: placebo + FLOT | • Primary endpoint: EFS<br>• Secondary endpoints: OS (Arm 1 vs. Arm 2) and pCR (Arm 1 vs. Arm 2) | • FPCD: Q4 2020<br>• LPCD: Q3 2022<br>• Data anticipated: H2 2025 |
| **Phase III<br>HIMALAYA<br>NCT03298451** | 1L HCC | 1324 | • Arm 1: *Imfinzi* + *Imjudo*<br>• Arm 2: *Imfinzi*<br>• Arm 3: sorafenib | • Primary endpoint: OS<br>• Secondary endpoints: PFS, TTP and ORR | • FPCD: Q4 2017<br>• LPCD: Q4 2019<br>• Data readout: Q4 2021 |
| **Phase III<br>EMERALD-3<br>NCT05301842** | Locoregional HCC | 525 | • Arm 1: TACE + T300 + D + lenvatanib<br>• Arm 2: TACE + T300 + D<br>• Arm 3: TACE | • Primary endpoint: PFS<br>• Secondary endpoint: OS | • FPCD: Q2 2022<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Imfinzi* (PD-L1 mAb)

## Lung cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>AEGEAN<br>NCT03800134** | Perioperative NSCLC patients, Stage II and III resected NSCLC (incl. EGFR/ALK-positive) | 800 | • Arm 1: *Imfinzi* + platinum-based chemotherapy<br>• Arm 2: placebo + platinum-based chemotherapy | • Primary endpoints: pCR snd EFS<br>• Secondary endpoints: mPR and DFS | • FPCD: Q1 2019<br>• Data readout: Q1 2023 |
| **Phase III<br>ADJUVANT BR.31<br>NCT02273375<br>Partnered (CCTG)** | Adjuvant NSCLC patients, Stage Ib (≥4cm) - Stage IIIa resected (incl. EGFR/ALK-positive) | 1360 | • Arm 1: *Imfinzi* mg/kg i.v. Q4W x 12 months<br>• Arm 2: placebo<br>• Global trial | • Primary endpoint: DFS<br>• Secondary endpoint: OS | • FPCD: Q1 2015<br>• LPCD: Q1 2020<br>• Data readout: Q2 2024 |
| **Phase III<br>PACIFIC-4<br>NCT03833154** | Imfinzi with SBRT in unresected, Stage I/II NSCLC | 630 | • Arm 1: *Imfinzi* i.v. Q4W with definitive SBRT<br>• Arm 2: placebo with definitive SBRT | • Primary endpoint: PFS<br>• Secondary endpoint: OS | • FPCD: Q2 2019<br>• Data anticipated: 2026 |
| **Phase III<br>PACIFIC-5<br>NCT03706690** | Unresected, locally advanced NSCLC | 360 | • Arm 1: *Imfinzi* i.v. Q4W following chemotherapy/RT<br>• Arm 2: placebo following chemotherapy/RT<br>• Global trial (ex-US with China focus) | • Primary endpoint: PFS<br>• Secondary endpoint: OS | • FPCD: Q1 2019<br>• LPCD: Q2 2022<br>• Data readout: Q3 2024 |
| **Phase III<br>PACIFIC-8<br>NCT05211895<br>Partnered (Arcus Biosciences)** | Unresected, locally advanced NSCLC | 860 | • Arm 1: *Imfinzi* + domvanalimab following chemotherapy/RT<br>• Arm 2: *Imfinzi* + placebo following chemotherapy/RT | • Primary endpoint: PFS<br>• Secondary endpoint: OS | • FPCD: Q1 2022<br>• Data anticipated: >2026 |
| **Phase III<br>ADRIATIC<br>NCT03703297** | Limited-stage SCLC 1L following platinum-based concurrent chemoradiation therapy | 600 | • Arm 1: *Imfinzi* + *Imjudo* (4 doses)<br>• Arm 2: *Imfinzi*<br>• Arm 3: placebo | • Primary endpoints: PFS and OS | • FPCD: Q4 2018<br>• Data readout: Q2 2024<br>• Primary endpoint met |
| **Phase III<br>PACIFIC-9<br>NCT05221840<br>Partnered (Innate)** | Patients with locally advanced (Stage III), unresectable NSCLC who have not progressed following platinum-based CRT | 999 | • Arm 1: *Imfinzi* + oleclumab<br>• Arm 2: *Imfinzi* + monalizumab + placebo<br>• Arm 3: *Imfinzi* + placebo | • Primary endoint: PFS<br>• Secondary endpoints: OS, ORR, DoR, PFS2 and TFST | • FPCD: Q2 2022<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Imfinzi* (PD-L1 mAb)

## Lung cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**HUDSON**<br>**NCT03334617** | NSCLC, patients who progressed on an anti-PD-1/PD-L1-containing therapy | 531 | • Open-label, biomarker-directed, multi-centre trial<br>• Module 1: *Imfinzi* + *Lynparza*<br>• Module 2: *Imfinzi* + danvatirsen<br>• Module 3: *Imfinzi* + ceralasertib<br>• Module 4: *Imfinzi* + vistusertib<br>• Module 5: *Imfinzi* + oleclumab<br>• Module 6: *Imfinzi* + *Enhertu*<br>• Module 7: *Imfinzi* + cediranib<br>• Module 8: ceralasertib<br>• Module 9: *Imfinzi* + ceralasertib<br>• Module 10: *Imfinzi* + ceralasertib<br>• Module 11: ceralasertib | • Primary endpoint: ORR<br>• Secondary endpoints: efficacy including OS, PFS, DCR, safety and tolerability and DoR | • FPCD: Q1 2018<br>• LPCD: Q3 2023<br>• Data readout: Q4 2024 |
| **Phase II**<br>**NeoCOAST-2**<br>**NCT05061550** | Early-stage, resectable NSCLC (Stage II to Stage IIIA) | 630 | • Open-label trial<br>• Arm 1: *Imfinzi* + oleclumab + platinum doublet chemotherapy<br>• Arm 2: *Imfinzi* + monalizumab + platinum doublet chemotherapy<br>• Arm 3: volrustomig + platinum doublet chemotherapy<br>• Arm 4: *Datroway* + single agent platinum chemotherapy<br>• Arm 5: AZD0171 + platinum doublet chemotherapy<br>• Arm 6: rilvegostomig + platinum doublet chemotherapy<br>• Arm 7: *Datroway* + rilvegostomig + single agent platinum chemotherapy | • Primary endpoints: pCR and safety | • FPCD: Q2 2022<br>• Data anticipated: >2026 |
| **Phase I/II**<br>**SCope-D1**<br>**NCT04870112** | NSCLC, SCLC | 18 | • Open-label, multi-centre trial<br>• s.c. *Imfinzi* | • Primary endpoints: PK parameters and safety | • FPCD: Q4 2021<br>• LPCD: Q2 2022<br>• Trial discontinued due to strategic portfolio prioritisation |



As of 6 February 2025.
Appendix: Glossary.

# *Imfinzi* (PD-L1 mAb)

## Other cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>POTOMAC<br>NCT03528694** | Non-muscle-invasive bladder cancer | 1018 | • Arm 1: BCG (induction + maintenance)<br>• Arm 2: *Imfinzi* + BCG (induction only)<br>• Arm 3: *Imfinzi* + BCG (induction + maintenance) | • Primary endpoint: DFS | • FPCD: Q2 2018<br>• LPCD: Q4 2020<br>• Data anticipated: H2 2025 |
| **Phase III<br>NIAGARA<br>NCT03732677** | Muscle-invasive bladder cancer eligible for cisplatin | 1063 | • Arm 1: *Imfinzi* in combination with gemcitabine + cisplatin, *Imfinzi* maintenance<br>• Arm 2: gemcitabine + cisplatin | • Co-primary endpoints: pCR and EFS | • FPCD: Q4 2018<br>• LPCD: Q3 2021<br>• Data readout: Q2 2024 |
| **Phase III<br>SAMETA<br>NCT05043090** | MET-driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma | 200 | • Arm 1: *Orpathys* + *Imfinzi*<br>• Arm 2: sunitinib<br>• Arm 3: *Imfinzi* monotherapy | • Primary endpoint: PFS<br>• Secondary endpoints: OS, ORR, DoR and DCR | • FPCD: Q4 2021<br>• Data anticipated: H2 2025 |
| **Phase III<br>NILE<br>NCT03682068** | 1L bladder cancer | 1246 | • Arm 1: *Imfinzi* + *Imjudo* + SoC<br>• Arm 2: *Imfinzi* + SoC<br>• Arm 3: SoC | • Primary endpoint: OS | • FPCD: Q4 2018<br>• LPCD: Q2 2021<br>• Data anticipated: H2 2025 |
| **Phase III<br>VOLGA<br>NCT04960709** | Muscle-invasive bladder cancer ineligible to cisplatin | 677 | • Arm 1: *Imfinzi* + *Imjudo* + enfortumab vedotin<br>• Arm 2: *Imfinzi* + enfortumab vedotin<br>• Arm 3: SoC cystectomy | • Primary endpoints: safety, EFS and pCR<br>• Secondary endpoint: OS | • FPCD: Q4 2021<br>• Data anticipated: H2 2025 |
| **Phase II<br>BEGONIA<br>NCT03742102** | 1L mTNBC | 243 | • Arm 1: *Imfinzi* + paclitaxel<br>• Arm 2: *Imfinzi* + paclitaxel + *Truqap*<br>• Arm 5: *Imfinzi* + paclitaxel + oleclumab<br>• Arm 6: *Imfinzi* + *Enhertu*<br>• Arm 7: *Imfinzi* + *Datroway*<br>• Arm 8: *Imfinzi* + *Datroway* (PD-L1-high)<br>• Global trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: ORR, PFS, DoR, OS, PK and ADA | • FPCD: Q1 2019<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# *Lynparza* (PARP inhibitor)

## Imfinzi combinations

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DUO-O**<br>**NCT03737643** | 1L advanced ovarian cancer | 1407 | • Non-tBRCAm (tumour BRCA) patients<br>• Arm 1: chemotherapy + bevacizumab + *Imfinzi* placebo followed by bevacizumab + *Imfinzi* placebo + *Lynparza* placebo<br>• Arm 2: chemotherapy + bevacizumab + *Imfinzi* followed by bevacizumab + *Imfinzi* + *Lynparza* placebo<br>• Arm 3: chemotherapy + bevacizumab + *Imfinzi* followed by bevacizumab + *Imfinzi* + *Lynparza*<br>• tBRCAm patients<br>• chemotherapy + bevacizumab (optional) + *Imfinzi* followed by bevacizumab (optional) + *Imfinzi* + *Lynparza*<br>• Global trial | • Primary endpoint: PFS<br>• Secondary endpoints: OS and PFS2 | • FPCD: Q1 2019<br>• LPCD: Q2 2023<br>• Data readout: Q2 2023<br>• Primary endpoint met |
| **Phase III**<br>**DUO-E**<br>**NCT04269200** | 1L advanced and recurrent endometrial cancer | 805 | • Arm 1: chemotherapy + *Imfinzi* placebo followed by *Imfinzi* placebo + *Lynparza* placebo<br>• Arm 2: chemotherapy + *Imfinzi* followed by *Imfinzi* + *Lynparza* placebo<br>• Arm 3: chemotherapy + *Imfinzi* followed by *Imfinzi* + *Lynparza*<br>• Global trial | • Primary endpoint: PFS<br>• Secondary endpoints: OS, PFS2, ORR and DoR | • FPCD: Q2 2020<br>• LPCD: Q2 2023<br>• Data readout: Q2 2023<br>• Primary endpoint met |



As of 6 February 2025.
Appendix: Glossary.

# *Lynparza* (PARP inhibitor)

## Other cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**MONO-OLA1**<br>**NCT04884360** | BRCAwt advanced ovarian cancer, 1L maintenance | 366 | • Arm 1: *Lynparza* BID 24-month duration<br>• Arm 2: placebo BID 24-month duration<br>• Global trial – 12 countries | • Primary endpoints: PFS (BRCAwt HRD-positive) and PFS (BRCAwt)<br>• Secondary endpoints: OS, TFST and PFS2 | • FPCD: Q3 2021<br>• LPCD: Q1 2024<br>• Data anticipated: H1 2025 |
| **Phase III**<br>**PROpel**<br>**NCT03732820** | 1L metastatic castration-resistant prostate cancer | 906 | • Arm 1: *Lynparza* + abiraterone<br>• Arm 2: placebo + abiraterone<br>• Global trial (including China) | • Primary endpoint: rPFS<br>• Secondary endpoint: OS | • FPCD: Q4 2018<br>• LPCD: Q3 2022<br>• Data readout: Q3 2021<br>• Primary endpoint met |



As of 6 February 2025.
Appendix: Glossary.

# *Enhertu* (trastuzumab deruxtecan, HER2 ADC)

## Breast cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DESTINY-Breast02**<br>**NCT03523585**<br>**Partnered (Daiichi Sankyo)** | HER2-positive, unresectable and/or metastatic breast cancer pretreated with prior SoC HER2 therapies including trastuzumab emtansine | 600 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine | • Primary endpoint: PFS<br>• Secondary endpoints: OS, ORR, DoR and CBR | • FPCD: Q3 2018<br>• LPCD: Q4 2020<br>• Data readout: Q3 2022<br>• Primary endpoint met |
| **Phase III**<br>**DESTINY-Breast03**<br>**NCT03529110**<br>**Partnered (Daiichi Sankyo)** | HER2-positive, unresectable and/or metastatic breast cancer previously treated with trastuzumab and taxane | 524 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: ado-trastuzumab emtansine | • Primary endpoint: PFS<br>• Secondary endpoints: OS, ORR, DoR and CBR | • FPCD: Q3 2018<br>• LPCD: Q2 2020<br>• Data readout: Q3 2021<br>• Primary endpoint met |
| **Phase III**<br>**DESTINY-Breast04**<br>**NCT03734029**<br>**Partnered (Daiichi Sankyo)** | HER2-low, unresectable and/or metastatic breast cancer | 557 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: physician's choice of SoC chemotherapy (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel) | • Primary endpoint: PFS<br>• Secondary endpoints: OS, DoR and ORR | • FPCD: Q4 2018<br>• LPCD: Q4 2020<br>• Data readout: Q1 2022<br>• Primary endpoint met |
| **Phase III**<br>**DESTINY-Breast05**<br>**NCT04622319**<br>**Partnered (Daiichi Sankyo)** | High-risk HER2-positive with residual invasive breast cancer following neoadjuvant therapy | 1600 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: ado-trastuzumab emtansine | • Primary endpoint: IDFS<br>• Secondary endpoints: DFS, OS, DRFI and BMFI | • FPCD: Q4 2020<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**DESTINY-Breast06**<br>**NCT04494425**<br>**Partnered (Daiichi Sankyo)** | HER2-low and -ultralow, HR+ breast cancer with disease progression on endocrine therapy in the metastatic setting | 866 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: investigator's choice SoC chemotherapy (capecitabine, paclitaxel, nab-paclitaxel) | • Primary endpoint: PFS<br>• Secondary endpoints: OS, DoR and ORR | • FPCD: Q3 2020<br>• LPCD: Q2 2023<br>• Data readout: Q2 2024 |
| **Phase III**<br>**DESTINY-Breast09**<br>**NCT04784715**<br>**Partnered (Daiichi Sankyo)** | HER2-positive, metastatic breast cancer with no prior therapy for advanced or metastatic disease | 1157 | • Randomised, parallel assignment<br>• Arm 1: *Enhertu* + placebo<br>• Arm 2: *Enhertu* + pertuzumab<br>• Arm 3: SoC | • Primary endpoint: PFS<br>• Secondary endpoints: OS, DoR and ORR | • FPCD: Q2 2021<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# *Enhertu* (trastuzumab deruxtecan, HER2 ADC)

## Breast cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DESTINY-Breast11**<br>**NCT05113251**<br>**Partnered (Daiichi Sankyo)** | High-risk HER2-positive early non-metastatic breast cancer | 927 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: *Enhertu* followed by THP<br>• Arm 3: doxorubicin and cyclophosphamide followed by THP | • Primary endpoint: pCR<br>• Secondary endpoints: EFS, IDFS and OS | • FPCD: Q4 2021<br>• Data anticipated: H1 2025 |
| **Phase Ib/II**<br>**DESTINY-Breast07**<br>**NCT04538742**<br>**Partnered (Daiichi Sankyo)** | HER2-positive metastatic breast cancer | 245 | • Randomised, open-label, sequential assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: *Enhertu* + *Imfinzi*<br>• Arm 3: *Enhertu* + pertuzumab<br>• Arm 4: *Enhertu* + paclitaxel<br>• Arm 5: *Enhertu* + *Imfinzi* + paclitaxel<br>• Arm 6: *Enhertu* + tucatinib | • Primary endpoints: AE and SAE<br>• Secondary endpoints: ORR, PFS, DoR and OS | • FPCD: Q1 2021<br>• Data anticipated: H1 2025 |
| **Phase Ib**<br>**DESTINY-Breast08**<br>**NCT04556773**<br>**Partnered (Daiichi Sankyo)** | HER2-low metastatic breast cancer | 139 | • Non-randomised, open-label parallel assignment<br>• Arm 1: *Enhertu* + capecitabine<br>• Arm 2: *Enhertu* + *Imfinzi* + paclitaxel<br>• Arm 3: *Enhertu* + *Truqap*<br>• Arm 4: *Enhertu* + anastrozole<br>• Arm 5: *Enhertu* + *Faslodex* | • Primary endpoints: AE and SAE<br>• Secondary endpoints: ORR, PFS, DoR and OS | • FPCD: Q1 2021<br>• LPCD: Q1 2023<br>• Data readout: Q3 2023 |



As of 6 February 2025.
Appendix: Glossary.

# *Enhertu* (trastuzumab deruxtecan, HER2 ADC)

## Gastric cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DESTINY-Gastric04**<br>**NCT04704934**<br>**Partnered (Daiichi Sankyo)** | HER2-positive gastric cancer or GEJ adenocarcinoma patients who have progressed on or after a trastuzumab-containing regimen and have not received any additional systemic therapy | 490 | • Open-label, randomised, parallel group assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: SoC chemotherapy | • Primary endpoint: OS<br>• Secondary endpoints: ORR, DoR, PFS, DcR and safety | • FPCD: Q2 2021<br>• Data anticipated: H2 2025 |
| **Phase II**<br>**DESTINY-Gastric06**<br>**NCT04989816**<br>**Partnered (Daiichi Sankyo)** | HER2-positive gastric cancer or GEJl junction adenocarcinoma patients who have progressed on two prior treatment regimens | 95 | • Open-label, single group assignment<br>• *Enhertu*<br>• China only | • Primary endpoint: ORR<br>• Secondary endpoints: PFS, ORR, DCR, OS, DoR and safety | • FPCD: Q3 2021<br>• LPCD: Q2 2024<br>• Data readout: Q3 2023<br>• |
| **Phase Ib/II**<br>**DESTINY-Gastric03**<br>**NCT04379596**<br>**Partnered (Daiichi Sankyo)** | Metastatic or unresectable HER2+ GC, GEJ, & esophageal adenocarcinoma<br>Part 1: ≥ 2L following trastuzumab containing therapy<br>Part 2, 3 and 4: Previously untreated metastatic or unresectable GC<br>Part 3 and 4: HER2 expressing (IHC 3+,2+,1+) | 417 | • Open-label, parallel assignment<br>• Part 1: to determine recommended Phase II combination dose<br>• 5 Arms combining *Enhertu* with SoC chemotherapies (5-FU, capecitabine, oxaliplatin) and/or durvalumab<br>• Part 2 and 3: to assess efficacy of the selected combinations<br>• Arm 2A: standard chemotherapy<br>• Arm 2B: *Enhertu* monotherapy<br>• Arm 2C: *Enhertu* with chemotherapy<br>• Arm 2D: *Enhertu* with chemotherapy and pembrolizumab<br>• Arm 2E: *Enhertu* and pembrolizumab<br>• Arm 2F: *Enhertu*, chemotherapy and pembrolizumab<br>• Arm 3A (HER2+): *Enhertu*, chemotherapy and volrustomig<br>• Arm 3B (HER2low): *Enhertu*, chemotherapy and volrustomig<br>• Arm 4A (HER2+): *Enhertu*, chemotherapy and rilvegostomig<br>• Arm 4B (HER2low): *Enhertu*, chemotherapy and rilvegostomig | • Primary endpoint (Part 1): safety, RP2D and ORR<br>• Secondary endpoints: DoR, DCR, PFS, OS, PK parameters and presence of ADAs | • FPCD: Q2 2020<br>• Data anticipated: 2026 |
| **Phase III**<br>**DESTINY-Gastric05**<br>**NCT06731478**<br>**Partnered (Daiichi Sankyo)** | HER2+ 1L locally advanced or metastatic GC or GEJ adenocarcinoma | 726 | • Arm A (CPS ≥1): *Enhertu* + 5-FU or capecitabine + pembrolizumab<br>• Arm B (CPS ≥1): *Enhertu* + 5-FU or capecitabine + cisplatin or oxaliplatin + pembrolizumab<br>• Arm C (CPS <1): *Enhertu* + 5-FU or capecitabine<br>• Arm D (CPS <1): ToGA | • Primary endpoint: PFS (BICR) in ITT<br>• Secondary endpoints: OS, ORR, PFS (Inv.), DOR, safety and PRO | • FPCD: Q1 2025<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Enhertu* (trastuzumab deruxtecan, HER2 ADC)

## Other cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DESTINY-Lung04**<br>**NCT05048797**<br>**Partnered (Daiichi Sankyo)** | *HER2*-mutated, unresectable, locally advanced/metastatic NSCLC | 450 | • Randomised, parallel group assignment<br>• Arm 1: *Enhertu*<br>• Arm 2: SoC (platinum, pemetrexed and pembrolizumab) | • Primary endpoint: PFS<br>• Secondary endpoints: OS, CNS-PFS, PFS (INV), ORR, DoR, safety, PK parameters, ADA, PRO-tolerability and PRO- pulmonary symptoms | • FPCD: Q4 2021<br>• Data anticipated: H2 2025 |
| **Phase II**<br>**DESTINY-Lung02**<br>**NCT04644237**<br>**Partnered (Daiichi Sankyo)** | HER2-mutated, unresectable and/or metastatic NSCLC | 152 | • Randomised, parallel group assignment<br>• Arm 1: *Enhertu* 6.4mg/kg<br>• Arm 2: *Enhertu* 5.4mg/kg | • Primary endpoint: ORR<br>• Secondary endpoints: DoR, DCR, PFS, OS and PK parameters | • FPCD: Q1 2021<br>• Data readout: Q1 2023<br>• Primary endpoint met |
| **Phase II**<br>**DESTINY-PanTumor02**<br>**NCT04482309**<br>**Partnered (Daiichi Sankyo)** | HER2-expressing tumours | 468 | • Non-randomised, single group assignment<br>• *Enhertu* | • Primary endpoint: ORR<br>• Secondary endpoints: DoR, DCR, PFS and OS | • FPCD: Q4 2020<br>• Data readout: Q3 2023 |
| **Phase II**<br>**DESTINY-PanTumor01**<br>**NCT04639219**<br>**Partnered (Daiichi Sankyo)** | HER2-mutated tumours | 102 | • Non-randomised, single group assignment<br>• *Enhertu* | • Primary endpoint: ORR<br>• Secondary endpoints: DoR, DCR, PFS and PK parameters | • FPCD: Q1 2021<br>• LPCD: Q2 2022<br>• Data readout: Q2 2023 |
| **Phase II**<br>**DESTINY-CRC02**<br>**NCT04744831**<br>**Partnered (Daiichi Sankyo)** | HER2-overexpressing advanced or metastatic colorectal cancer | 122 | • Randomised, parallel group assignment<br>• Arm 1: *Enhertu* 6.4mg/kg<br>• Arm 2: *Enhertu* 5.4mg/kg | • Primary endpoint: ORR<br>• Secondary endpoints: ORR, PFS, OS, DoR, DCR and PK parameters | • FPCD: Q1 2021<br>• Data readout: Q1 2023<br>• Primary endpoint met |



As of 6 February 2025.
Appendix: Glossary.

# *Enhertu* (trastuzumab deruxtecan, HER2 ADC)

## Other cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase Ib**<br>**DESTINY-Lung03**<br>**NCT04686305**<br>**Partnered (Daiichi Sankyo)** | HER2-overexpressing, unresectable and/or metastatic NSCLC<br>Part 1: 2L/3L advanced<br>Parts 2/3/4: 1L advanced | 244 | • Non-randomised, parallel group assignment<br>• Part 1: to determine recommended combination dose<br>• 3 Arms combine *Enhertu* with SoC chemotherapies (cisplatin, carboplatin or pemetrexed) and *Imfinzi*; Arm 1D: *Enhertu* monotherapy arm<br>• Part 2: to assess efficacy of the selected combinations with chemotherapies (cisplatin, carboplatin or pemetrexed) and *Imfinzi* not initiated<br>• Part 3 (2 arms): dose confirmation to assess safety and efficacy with volrustomig and volrustomig and chemotherapy (carboplatin)<br>• Part 4 (2 arms): dose confirmation to assess safety and efficacy with rilvegostomig and rilvegostomig and chemotheraoy (carboplatin) | • Primary endpoint: safety and RP2D<br>• Secondary endpoints: ORR, DoR, DCR, PFS, OS and PK parameters | • FPCD: Q4 2021<br>• Data anticipated: 2026 |
| **Phase Ib**<br>**U106**<br>**NCT04042701**<br>**Partnered (Daiichi Sankyo)** | HER2-over expressing locally advanced/metastatic breast or NSCLC | 115 | • Non-randomised, parallel group assignment<br>• *Enhertu* + pembrolizumab<br>• Global trial – 2 countries | • Primary endpoints: DLT and ORR<br>• Secondary endpoints: DoR, DCR, PFS, TTR and OS | • FPCD: Q2 2020<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**DESTINY-BTC01**<br>**NCT06467357**<br>**Partnered (Daiichi Sankyo)** | Advanced treatment-naïve HER2-expressing BTC | 620 | • Arm A: *Enhertu* + rilvegostomig<br>• Arm B: *Enhertu*<br>• Arm C: gemcitabine and cisplatin + Imfinzi | • Primary endpoint: OS<br>• Secondary endpoint: OS (ITT), PFS (INV), ORR (ONV), DOR (INV) Safety, PRO | • FPCD: Q3 2024<br>• Data anticipated: >2026 |
| **Phase II**<br>**DESTINY-PanTumor03**<br>**NCT06271837**<br>**Partnered (Daiichi Sankyo)** | HER2 expressing tumours | 125 | • Non-randomised single group assignment<br>• *Enhertu*<br>• China only | • Primary endpoint: ORR<br>• Secondary endpoints: DoR, DCR, PFS, OS, safety and tolerability, PK | • FPCD: Q3 2024<br>• Data anticipated: H2 2025 |
| **Phase II**<br>**DESTINY-Lung05**<br>**NCT05246514**<br>**Partnered (Daiichi Sankyo)** | *HER2*-mutant metastatic NSCLC who have disease progression on or after at least one-line of treatment | 80 | • Open-label, single-arm trial<br>• China only | • Primary endpoint: ORR<br>• Secondary endpoints: investigator and ICR assessed DCR, DoR and PFS, investigator assessed ORR, OS, ICR assessed NS-PFS, PK parameters, immunogenicity and safety | • FPCD: Q3 2022<br>• LPCD: Q1 2023<br>• Data readout: Q4 2023<br>• Primary endpoint met |



As of 6 February 2025.
Appendix: Glossary.

# *Calquence* (BTK inhibitor)

## Blood cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**AMPLIFY (ACE-CL-311)**<br>**NCT03836261** | Previously untreated CLL | 981 | • Arm 1; *Calquence* + venetoclax<br>• Arm 2: *Calquence* + venetoclax + obinutuzumab<br>• Arm 3: FCR or BR | • Primary endpoint: IRC PFS (Arm 1 vs. Arm 3)<br>• Secondary endpoints: IRC PFS (Arm 2 vs. Arm 3) and INV PFS (Arm 1 vs. Arm 3; Arm 2 vs. Arm 3) | • FPCD: Q1 2019<br>• LPCD: Q3 2023<br>• Data readout: Q3 2024<br>• Primary endpoint met |
| **Phase III**<br>**ECHO (ACE-LY-308)**<br>**NCT02972840** | Previously untreated MCL | 634 | • Arm 1: *Calquence* + bendamustine + rituximab<br>• Arm 2: bendamustine + rituximab | • Primary endpoint: PFS by Lugano Classification for NHL<br>• Secondary endpoints: IA, PFS, ORR, DoR, time to response and OS | • FPCD: Q2 2017<br>• LPCD: 1Q 2023<br>• Data readout: Q2 2024<br>• Primary endpoint met |
| **Phase III**<br>**ESCALADE**<br>**NCT04529772** | DLBCL | 600 | • *Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone | • Primary endpoint: PFS | • FPCD: Q1 2021<br>• Data anticipated: >2026 |
| **Phase III**<br>**NCT04075292** | Untreated CLL | 155 | • Arm 1: *Calquence*<br>• Arm 2: chlorambucil + rituximab | • Primary endpoint: PFS<br>• Secondary endpoints: ORR and DoR | • FPCD: Q1 2020<br>• Data readout: Q2 2024 |
| **Phase II**<br>**TrAVeRse**<br>**NCT05951959** | Treatment-naïve MCL | 100 | • Open-label, single-arm trial<br>• *Calquence* + venetoclax + rituximab | • Primary endpoint: MRD-negative CR at end of induction | • FPCD: Q1 2024<br>• Data anticipated: >2026 |
| **Phase Ib**<br>**ACE-LY-106**<br>**NCT02717624** | MCL | 61 | • *Calquence* in combination with bendamustine and rituximab<br>• Arm 1: treatment naive<br>• Arm 2: R/R<br>• Arm 3: treatment naïve: *Calquence* + venetoclax + rituximab | • Primary endpoint: safety | • FPCD: Q2 2016<br>• LPCD: Q2 2022<br>• Data readout: Q1 2023 |
| **Phase I**<br>**ACE-LY-003**<br>**NCT02180711** | R/R follicular lymphoma | 89 | • Arm 1: *Calquence*<br>• Arm 2: *Calquence* + rituximab<br>• Arm 3: *Calquence* + rituximab + lenolidomide | • Primary endpoint: safety | • FPCD: Q1 2015<br>• LPCD: Q4 2021<br>• Data readout: Q1 2024 |



As of 6 February 2025.
Appendix: Glossary.

# *Orpathys* (savolitinib, MET inhibitor)

## NSCLC and other cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**NCT04923945**<br>**Partnered (HUTCHMED)** | Locally advanced or metastatic NSCLC patients with MET exon 14 mutations without EGFR, ALK and ROS1 mutations progressing on platinum chemotherapy and are treatment naïve to c-MET therapy or did not receive prior drug therapy for advanced tumours | 163 | • Single-arm trial<br>• *Orpathys* | • Primary endpoint: ORR | • FPCD: Q3 2021<br>• LPCD: Q2 2023<br>• Data readout: Q4 2024 |
| **Phase II**<br>**NCT04923932**<br>**Partnered (HUTCHMED)** | Locally advanced or metastatic gastric cancer and esophagogastric junction adenocarcinoma patients with MET gene amplifications | 75 | • Single-arm, multi-cohort, multi-centre, open-label trial<br>• *Orpathys* | • Primary endpoint: ORR<br>• Secondary endpoints: PFS and safety | • FPCD: Q3 2021<br>• Data anticipated: H1 2025 |



As of 6 February 2025.
Appendix: Glossary.

# *Tagrisso* (highly-selective, irreversible EGFR inhibitor)

## NSCLC

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**LAURA**<br>**NCT03521154** | Maintenance therapy in patients with locally advanced, unresectable *EGFR*m Stage III NSCLC whose disease has not progressed following platinum-based chemoradiation therapy | 216 | • Arm 1: *Tagrisso*<br>• Arm 2: placebo<br>• Global trial – 17 countries | • Primary endpoint: PFS (BICR)<br>• Secondary endpoints: CNS PFS, OS, DoR, ORR and DCR | • FPCD: Q4 2018<br>• LPCD: Q3 2022<br>• Data readout: Q1 2024<br>• Primary endpoint met |
| **Phase III**<br>**ADAURA2**<br>**NCT05120349** | Adjuvant EGFRm NSCLC Stage IA2 to IA3 following complete tumour resection | 380 | • Arm 1: *Tagrisso*<br>• Arm 2: placebo | • Primary endpoint: DFS<br>• Secondary endpoints: DFS rate, OS, OS rate and QoL | • FPCD: Q2 2022<br>• LPCD: Q4 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Tagrisso* (highly-selective, irreversible EGFR inhibitor)

## NSCLC, combinations

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>NeoADAURA<br>NCT04351555** | Neoadjuvant *EGFR*m NSCLC | 351 | • Arm 1: placebo + pemetrexed/carboplatin or pemetrexed/cisplatin<br>• Arm 2: *Tagrisso* + pemetrexed/carboplatin or pemetrexed/cisplatin<br>• Arm 3: *Tagrisso*<br>• Global trial – 23 countries | • Primary endpoint: mPR<br>• Secondary endpoints: cPR, EFS, DFS and OS | • FPCD: Q1 2021<br>• LPCD: Q4 2023<br>• Data readout: Q4 2024<br>• Primary endpoint met |
| **Phase III<br>SAFFRON<br>NCT05261399<br>Partnered (HUTCHMED)** | *EGFR*m, MET-overexpressed and/or amplified, locally advanced or metastatic NSCLC patients who have progressed on first- or second-line treatment with *Tagrisso* | 324 | • Arm 1: *Tagrisso* + *Orpathys*<br>• Arm2: pemetrexed with either cisplatin or carboplatin | • Primary enpoint: PFS<br>• Secondary endpoints: OS, ORR, PK, DCR and DoR | • FPCD: Q3 2022<br>• Data anticipated: H2 2025 |
| **Phase III<br>SANOVO<br>NCT05009836<br>Partnered (HUTCHMED)** | 1L *EGFR*m, MET+ locally advanced or metastatic NSCLC | 320 | • Arm 1: *Tagrisso* + *Orpathys*<br>• Arm 2: *Tagrisso* + placebo | • Primary endpoint: PFS | • FPCD: Q3 2021<br>• Data anticipated: H2 2025 |
| **Phase III<br>SACHI<br>NCT05015608<br>Partnered (HUTCHMED)** | Locally advanced or metastatic NSCLC with MET amplification after failure of the first-line EGFR inhibitor therapy | 250 | • Arm 1: *Tagrisso* + *Orpathys*<br>• Arm 2: pemetrexed + platinum<br>• China only | • Primary endpoint: PFS | • FPCD: Q3 2021<br>• Data readout: Q3 2024<br>• Primary endpoint met |



As of 6 February 2025.
Appendix: Glossary.

# *Tagrisso* (highly-selective, irreversible EGFR inhibitor)

## NSCLC, combinations

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**SAVANNAH**<br>**NCT03778229**<br>**Partnered (HUTCHMED)** | *EGFR*m/MET+, locally advanced or metastatic NSCLC who have progressed following treatment with Tagrisso | 360 | • Protocol v1-6: single-arm, open-label trial<br>• Protocol v7: randomised, double-blind trial<br>• Arm 1: *Tagrisso* + *Orpathys*<br>• Arm 2: placebo + *Orpathys*<br>• Global trial | • Primary endpoint: ORR<br>• Secondary endpoints: PFS, DoR and OS | • FPCD: Q1 2019<br>• LPCD: Q1 2024<br>• Data readout: Q3 2024<br>• Clinically meaningful ORR |
| **Phase II**<br>**ORCHARD**<br>**NCT03944772** | Advanced EGFRm NSCLC patients who have progressed on first-line Tagrisso treatment | 250 | • Modular design platform trial:<br>• Module 1: *Tagrisso* + *Orpathys* (cMET)<br>• Module 2: *Tagrisso* + gefitinib (*EGFR*m)<br>• Module 3: *Tagrisso* + necitumumab (EGFRm)<br>• Module 4: carboplatin + pemetrexed + *Imfinzi*<br>• Module 5: *Tagrisso* + alectinib (ALK)<br>• Module 6: *Tagrisso* + selpercatinib (RET fusion)<br>• Module 7: *Imfinzi* + etoposide + carboplatin or cisplatin<br>• Module 8: *Tagrisso* + pemetrexed + carboplatin or cisplatin<br>• Module 9: *Tagrisso* + *Koselugo*<br>• Module 10: *Tagrisso* + *Datroway*<br>• No intervention: observational cohort<br>• Global trial | • Primary endpoint: ORR<br>• Secondary endpoints: PFS, DoR, OS, safety and tolerability | • FPCD: Q3 2019<br>• LPCD: Q4 2023<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# *Truqap* (capivasertib, AKT inhibitor)

## Breast cancer and prostate cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**CAPItello-291**<br>**NCT04305496** | 2L+ AI-resistant locally advanced (inoperable) or metastatic HR+ HER2-negative breast cancer | 834 | • Double-blind, randomised, comparative trial<br>• Arm 1: *Truqap* + *Faslodex*<br>• Arm 2: placebo + *Faslodex* | • Primary endpoint: PFS | • FPCD: Q2 2020<br>• LPCD: Q4 2021<br>• Data readout: Q4 2022<br>• Both primary endpoints met |
| **Phase III**<br>**CAPItello-281**<br>**NCT04493853** | De novo *PTEN* deficient metastatic hormone sensitive prostate cancer | 1000 | • Double-blind, randomised, comparative trial<br>• Arm 1: *Truqap* + abiraterone<br>• Arm 2: placebo + abiraterone | • Primary endpoint: rPFS | • FPCD: Q3 2020<br>• LPCD: Q1 2024<br>• Data readout: Q4 2024<br>• Primary endpoint met |
| **Phase III**<br>**CAPItello-280**<br>**NCT05348577** | mCRPC prostate cancer | 790 | • Double-blind, randomised, comparative trial<br>• Arm 1: *Truqap* + docetaxel<br>• Arm 2: placebo + docetaxel | • Primary endpoint: OS | • FPCD: Q2 2022<br>• LPCD: Q3 2024<br>• Data anticipated: 2026 |
| **Phase Ib/III**<br>**CAPItello-292**<br>**NCT04862663** | 1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+ HER2-negative breast cancer | 700 | • Double-blind, randomised, comparative trial<br>• Arm 1: *Truqap* + palbociclib + *Faslodex*<br>• Arm 2: placebo + palbociclib + *Faslodex* | • Primary endpoint: PFS | • FPCD: Q2 2021<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Datroway* (datopotamab deruxtecan, TROP2 ADC)

## Breast cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**TROPION-Breast01**<br>**NCT05104866**<br>**Partnered (Daiichi Sankyo)** | Inoperable or metastatic HR+ HER2- breast cancer after treatment with one or two prior lines of systemic chemotherapy | 732 | • Open-label, randomised trial<br>• Arm 1: *Datroway*<br>• Arm 2: investigator's choice SoC chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine)<br>• Global trial | • Primary endpoints: PFS (BICR) and OS<br>• Secondary endpoints: ORR, DoR, PFS (Inv), DCR, PK parameters and ADA | • FPCD: Q4 2021<br>• LPCD: Q4 2022<br>• Data readout: Q3 2023<br>• Dual primary endpoint (OS) not met |
| **Phase III**<br>**TROPION-Breast02**<br>**NCT05374512**<br>**Partnered (Daiichi Sankyo)** | Locally recurrent inoperable or metastatic TNBC | 600 | • Open-label, randomised trial<br>• Arm 1: *Datroway*<br>• Arm 2: investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate)<br>• Global trial | • Primary endpoints: PFS (BICR) and OS<br>• Secondary endpoints: PFS (Inv), ORR, DoR, PK parameters and ADA | • FPCD: Q2 2022<br>• Data anticipated: H1 2025 |
| **Phase III**<br>**TROPION-Breast03**<br>**NCT05629585**<br>**Partnered (Daiichi Sankyo)** | Stage I-III TNBC without pathological complete response following neoadjuvant therapy | 1075 | • Open-label, randomised trial<br>• Arm 1: *Datroway* + *Imfinzi*<br>• Arm 2: *Datroway*<br>• Arm 3: investigator's choice of therapy (capecitabine, pembrolizumab, or capecitabine + pembrolizumab)<br>• Global trial | • Primary endpoint: iDFS<br>• Secondary endpoints: DDFS, OS, PK parameters and ADA | • FPCD: Q4 2022<br>• Data anticipated: 2026 |
| **Phase III**<br>**TROPION-Breast04**<br>**NCT06112379**<br>**Partnered (Daiichi Sankyo)** | Perioperative triple-negative or HR-low/HER2-negative breast cancer | 1728 | • Open-label, randomised<br>• Arm 1: *Datroway* + *Imfinzi*<br>• Arm 2: pembrolizumab + chemotherapy<br>• Global trial | • Dual primary endpoint: pCR and EFS<br>• Secondary endpoints: OS, DDFS and safety | • FPCD: Q4 2023<br>• Data anticipated: >2026 |
| **Phase III**<br>**TROPION-Breast05**<br>**NCT06103864**<br>**Partnered (Daiichi Sankyo)** | Patients with PD-L1-positive locally recurrent inoperable or metastatic TNBC | 625 | • Open-label, randomised<br>• Arm 1: *Datroway* + *Imfinzi*<br>• Arm 2: investigator's choice of chemotherapy in combination with pembrolizumab (paclitaxel, nab-paclitaxel, or gemcitabine + carboplatin)<br>• Arm 3: *Datroway*<br>• Global trial | • Primary endpoint: PFS (BICR)<br>• Secondary endpoints: OS, PFS (inv), ORR, DoR, DCR and safety | • FPCD: Q4 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Datroway* (datopotamab deruxtecan, TROP2 ADC)

## NSCLC

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>AVANZAR<br>NCT05687266** | 1L NSCLC | 1350 | • Arm 1: carboplatin + *Datroway* + *Imfinzi*<br>• Arm 2: pembrolizumab<br>• Global trial | • Co-primary endpoints: PFS and OS in NSQ ITT and NSQ TROP2 biomarker-positive | • FPCD: Q1 2023<br>• Data anticipated: H2 2025 |
| **Phase III<br>TROPION-Lung01<br>NCT04656652<br>Partnered (Daiichi Sankyo)** | Previously treated advanced or metastatic NSCLC with or without actionable genomic alterations | 590 | • Randomised, open-label, parallel assignment<br>• Arm 1: *Datroway*<br>• Arm 2: docetaxel<br>• Global trial | • Primary endpoints: PFS and OS<br>• Secondary endpoints: ORR, DoR, TTR, DCR, PK parameters and ADA | • FPCD: Q1 2021<br>• LPCD: Q4 2022<br>• Data readout: Q3 2023<br>• Dual primary endpoint met (PFS) |
| **Phase III<br>TROPION-Lung07<br>NCT05555732<br>Partnered (Daiichi Sankyo)** | 1L patients with PD-L1 TPS <50% and advanced or metastatic NSCLC without actionable genomic alternations | 1170 | • Randomised, open-label<br>• Arm 1: *Datroway* + pembrolizumab + platinum chemotherapy<br>• Arm 2: *Datroway* + pembrolizumab<br>• Arm 3: pembrolizumab + pemetrexed + platinum chemotherapy<br>• Global trial | • Primary endpoints: PFS and OS | • FPCD: Q1 2023<br>• Data anticipated: 2026 |
| **Phase III<br>TROPION-Lung08<br>NCT05215340<br>Partnered (Daiichi Sankyo)** | Treatment-naïve patients with PD-L1-high advanced or metastatic NSCLC without actionable genomic alterations | 740 | • Randomised, open-label<br>• Arm 1: *Datroway* + pembrolizumab<br>• Arm 2: pembrolizumab<br>• Global trial | • Primary endpoints: PFS and OS | • FPCD: Q1 2022<br>• Data anticipated: 2026 |
| **Phase III<br>TROPION-Lung10<br>NCT06357533<br>Partnered (Daiichi Sankyo)** | Locally advanced or metastatic non-squamous NSCLC with high PD-L1 expression (TC ≥50%) and without actionable genomic alterations | 675 | • Randomised, open-label, sponsor-blinded, parallel assignment<br>• Arm 1: *Datroway* + rilvegostomig<br>• Arm 2: rilvegostomig<br>• Arm 3: pembrolizumab | • Primary endpoints: PFS and OS in TROP2 biomarker-positive participants<br>• Secondary endpoints: PFS and OS in the ITT population, ORR, DoR, TTD, PK parameters, immunogenicity and PFS2 | • FPCD: Q2 2024<br>• Data anticipated: >2026 |
| **Phase III<br>TROPION-Lung12<br>NCT06564844<br>Partnered (Daiichi Sankyo)** | Stage I adenocarcinoma NSCLC who are ctDNA-positive or have high-risk pathological features | 660 | • Randomised trial<br>• Arm 1: *Datroway* + rilvegostomig<br>• Arm 2: rilvegostomig<br>• Arm 3: standard of care | • Primary endpoint: DFS (BICR)<br>• Secondary endpoint: OS, QoL and PK parameters | • FPCD: Q4 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Datroway* (datopotamab deruxtecan, TROP2 ADC)

## NSCLC

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**TROPION-Lung14**<br>**NCT06350097**<br>**Partnered (Diachii Sankyo)** | *EGFR*m locally advanced or metastatic NSCLC | 562 | • Arm 1: *Tagrisso* + *Datroway*<br>• Arm 2: *Tagrisso* monotherapy | • Primary endpoint: PFS (BICR)<br>• Secondary endpoints: OS, PFS by Inv., ORR, DoR; DCR; PFS of CNS met. patients; PFS2; safety; PK parameters and immunogenicity | • FPCD: Q2 2024<br>• Data anticipated: >2026 |
| **Phase III**<br>**TROPION-Lung15**<br>**NCT06417814**<br>**Partnered (Daiichi Sankyo)** | Patients with advanced or metastatic EGFRm NSCLC whose disease has progressed on prior *Tagrisso* | 630 | • Open-label, sponsor blind, randomised trial<br>• Arm 1: Datroway + Tagrisso<br>• Arm 2: Datroway<br>• Arm 3: Platinum-based doublet CTx | • Dual primary endpoints: PFS (BICR) monotherapy vs. CTx and PFS (BICR) combination vs. CTx<br>• Secondary endpoints: OS, CNS PFS, PFS (Inv.), PFS2, ORR, DoR, DCR, TTR, safety and PRO | • FPCD: Q4 2024<br>• Data anticipated: 2026 |
| **Phase II**<br>**TROPION-Lung05**<br>**NCT04484142**<br>**Partnered (Daiichi Sankyo)** | Advanced or metastatic NSCLC with actionable genomic alterations and progressed on or after kinase inhibitor therapy and platinum-based chemotherapy | 137 | • Single-arm, open-label<br>• *Datroway*<br>• Global trial | • Primary endpoint: ORR<br>• Secondary endpoints: DOR, PFS, OS, safety, PK parameters and ADA | • FPCD: Q1 2021<br>• LPCD: Q1 2022<br>• Data anticipated: H2 2025 |
| **Phase I**<br>**TROPION-Lung02**<br>**NCT04526691**<br>**Partnered (Daiichi Sankyo)** | Advanced or metastatic NSCLC | 145 | • Open-label, two-part (dose escalation and dose expansion), sequential assignment<br>• *Datroway* + pembrolizumab +/- platinum chemotherapy<br>• Global trial – US, Japan, Italy, Spain and Taiwan | • Primary endpoints: DLT and safety<br>• Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA | • FPCD: Q4 2020<br>• LPCD: Q2 2023<br>• Data readout: Q4 2024 |
| **Phase I**<br>**TROPION-Lung04**<br>**NCT04612751**<br>**Partnered (Daiichi Sankyo)** | Advanced or metastatic NSCLC | 371 | • Open-label, two-part (dose escalation, dose expansion), sequential assignment<br>• *Datroway* + *Imfinzi* +/- platinum chemotherapy<br>• Cohort 1 & 2: *Datroway* + *Imfinzi*<br>• Cohort 3 & 4: *Datroway* + *Imfinzi* + carboplatin<br>• Cohort 4a: *Datroway* + *Imfinzi* + carboplatin (SQ 1L only)<br>• Cohort 5 & 6: *Datroway* + rilvegostomig<br>• Cohort 7 & 8: *Datroway* + rilvegostomig + carboplatin<br>• Cohort 9 & 10: *Datroway* + volrustomig + carboplatin<br>• Cohort 11: *Datroway* + volrustomig<br>• Global trial | • Primary endpoints: DLT and safety<br>• Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA | • FPCD: Q1 2021<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Datroway* (datopotamab deruxtecan, TROP2 ADC)

## Other cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**TROPION-PanTumor03**<br>**NCT05489211**<br>**Partnered (Daiichi Sankyo)** | Endometrial cancer, gastric cancer, mCRPC, ovarian cancer, CRC, bladder cancer and BTC | 556 | • Sub-study 1 (endometrial cancer);<br>• Sub-study 1a: *Datroway* monotherapy<br>• Sub-study 2 (gastric cancer);<br>• Sub-study 2a: *Datroway* + capecitabine<br>• Sub-study 2b: *Datroway* + 5-fluorouracil<br>• Sub-study 3 (mCRPC);<br>• Sub-study 3a: *Datroway* (post-NHA)<br>• Sub-study 3c: *Datroway* + prednisone/prednisolone<br>• Sub-study 4 (ovarian cancer);<br>• Sub-study 4a: *Datroway*<br>• Sub-study 4a (expansion): *Datroway* PSR/PRR (2-3L)<br>• Sub-study 4c: *Datroway* + carboplatin + bevacizumab PSR (2-3L)<br>• Sub-study 5 (CRC);<br>• Sub-study 5a1: *Datroway* (TROP2+ 3L+)<br>• Sub-study 5a2: *Datroway* (TROP2+ 2L+)<br>• Sub-study 5b: *Datroway* + 5-FU/leucovorin or Capecitabine + bevacizumab (TROP2+ 1L)<br>• Sub-study 6 (bladder);<br>• Sub-study 6d: *Datroway* (2L+)<br>• Sub-study 6b: 1L cis-ineligible/2L *Datroway* + rilvegostomig (1L)<br>• Sub-study 6c: post-pembro/EV - *Datroway* + carbo/cisplatin (2L)<br>• Sub-study 7 (BTC)<br>• Sub-study 7a: TROP2+ *Datroway* (2L+) | • Primary endpoints: ORR and safety | • FPCD: Q3 2022<br>• Data anticipated: >2026 |
| **Phase I/II**<br>**TROPION-PanTumor02**<br>**NCT05460273**<br>**Partnered (Daiichi Sankyo)** | NSCLC and TNBC and other solid tumours in Chinese patients | 119 | • Single-arm, multi-cohort trial with no blinding<br>• *Datroway*<br>• China only | • Primary endpoint: ORR<br>• Secondary endpoints: DoR, DCR, BOR, TTR PFS and OS | • FPCD: Q3 2022<br>• LPCD: Q2 2023<br>• Data readout: Q2 2024 |
| **Phase I**<br>**TROPION-PanTumor01**<br>**NCT03401385**<br>**Partnered (Daiichi Sankyo)** | Subjects with advanced solid tumours: NSCLC, TNBC, HR+ breast cancer, HER2-negative gastric/GEJ, oesophageal, urothelial, SCLC, CRPC, PDAC, HNSCC, HR+ HER2-low breast cancer and HER2-positive breast cancer | 890 | • Open-label, two-part (dose escalation, dose expansion), sequential assignment<br>• *Datroway*<br>• US and Japan | • Primary endpoints: DLT and safety<br>• Secondary endpoints: PK parameters, anti-tumour activity and ADA | • FPCD: Q1 2018<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# AZD0486 (CD19/CD3 next-generation bispecific T-cell engager)

## Haematologic malignancies

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**SOUNDTRACK-F1**<br>**NCT06549595** | Previously untreated follicular lymphoma | 1005 | • Multi-centre, randomised, open-label trial<br>• Arm 1: rituximab + AZD0486 followed by observation<br>• Arm 2: rituximab + AZD0486 followed by maintenance AZD0486<br>• Arm 3: Investigator's choice of RCHOP/RCVP/BR followed by standard of care maintenance or observation | • Primary endpoint: PFS<br>• Secondary endpoint: CR | • FPCD: Q3 2024<br>• Data anticipated: >2026 |
| **Phase II**<br>**SOUNDTRACK-B**<br>**NCT06526793** | B-cell non-Hodgkin lymphoma, follicular lymphoma and diffuse large B-cell lymphoma | 240 | • Multi-centre, single-arm, open-label trial<br>• Sub-study 1 (RR CLL/SLL): AZD0486 +/- Calquence<br>• Sub-study 2 (RR MCL): AZD0486 +/- Calquence<br>• Sub-study 3 (RR LBCL): AZD0486 + R-CHOP | • Primary endpoint: ORR<br>• Secondary endpoints: DoR, CR and PFS | • FPCD: Q4 2024<br>• Data anticipated: >2026 |
| **Phase I/II**<br>**SYRUS**<br>**NCT06137118** | R/R B-ALL | 120 | • Multi-centre, open-label, single-arm dose escalation and dose optimisation trial | • Primary endpoints: DLT, safety and ORR<br>• Secondary endpoints: ORR, DoR, CR rate at any time during trial, EFS, OS, subsequent alloSCT, CR MRD-negative rate, PK parameters and ADA | • FPCD: Q1 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT04594642** | R/R B-cell non-Hodgkin lymphoma | 231 | • Multi-centre, open-label, dose escalation and dose expansion trial | • Primary endpoints: safety and tolerability, MTD and/or RP2D and PK parameters<br>• Secondary endpoints: clinical activity of AZD0486 monotherapy and ADA titers for AZD0486 monotherapy | • FPCD: Q1 2021<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD0901 (CLDN18.2 MMAE ADC)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**CLARITY- Gastric 01**<br>**NCT06346392** | 2L+ advanced or metastatic gastric or GEJ adenocarcinoma expressing CLDN18.2 | 589 | • Multi-centre, open-label, sponsor-blinded, randomised trial<br>• Arm 1: AZD0901 dose level 1 via i.v. infusion treatment<br>• Arm 2: AZD0901 dose level 2 via i.v. infusion treatment<br>• Arm 3: investigator's choice chemotherapies<br>• Global trial | • Primary endpoints: PFS and OS<br>• Secondary endpoints: OS, PFS for 3L+, ORR, ORR for 3L+, DoR, MMAE, safety and tolerability, PK parameters and prevalence of ADAs | • FPCD: Q2 2024<br>• Data anticipated: 2026 |
| **Phase II**<br>**NCT06219941** | Locally advanced unresectable or metastatic solid tumours expressing CLDN18.2 | 123 | • Open-label, multi-centre trial of AZD0901 administered via i.v.<br>• Sub-study 1: AZD0901 monotherapy<br>• Sub-study 2: AZD0901 and anti-cancer agents | • Primary endpoints: AEs, SAEs and ORR<br>• Secondary endpoints: OS, PFS, DoR, DCR, PK parameters and prevalence of ADAs | • FPCD: Q1 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# camizestrant (AZD9833, next-generation oral SERD)

## Breast cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III SERENA-4 NCT04711252** | HR+ HER2-negative advanced breast cancer | 1370 | • Randomised, double-blind, comparative trial<br>• Arm 1: camizestrant + palbociclib<br>• Arm 2: anastrazole + palbociclib | • Primary endpoint: PFS<br>• Secondary endpoints: OS and PFS2 | • FPCD: Q1 2021<br>• LPCD: Q4 2023<br>• Data anticipated: 2026 |
| **Phase III SERENA-6 NCT04964934** | HR+ HER2-negative advanced breast cancer | 312 | • Randomised, double-blind, comparator trial<br>• Arm 1: camizestrant + palbociclib or abemaciclib or ribociclib<br>• Arm 2: anastrazole or letrozole + palbociclib or abemaciclib or ribociclib | • Primary endpoint: PFS<br>• Secondary endpoint: OS and PFS2 | • FPCD: Q3 2021<br>• LPCD: Q3 2024<br>• Data anticipated: H2 2025 |
| **Phase III CAMBRIA-1 NCT05774951** | ER+/HER2-negative early breast cancer patients who completed definitive locoregional therapy and standard adjuvant ET for at least 2 years and up to 5 years | 4300 | • Arm 1: continue standard ET of investigator's choice<br>• Arm 2: camizestrant<br>• Global trial | • Primary endpoint: IBCFS<br>• Secondary endpoints: IDFS, DRFS and OS | • FPCD: Q1 2023<br>• Data anticipated: >2026 |
| **Phase III CAMBRIA-2 NCT05952557** | ER+/HER2-negative early breast cancer with intermediate-high or high risk of recurrence that has completed definitive locoregional therapy and have no evidence of disease | 5500 | • Arm A: standard endocrine therapy of investigator's choice (aromatase inhibitors [exemestane, letrozole, anastrozole] or tamoxifen) ± abemaciclib<br>• Arm B: camizestrant ± abemaciclib<br>• Global trial | • Primary endpoint: IBCFS<br>• Secondary endpoints: IDFS, DRFS and OS | • FPCD: Q4 2023<br>• Data anticipated: >2026 |
| **Phase II SERENA-2 NCT04214288** | HR+ advanced breast cancer | 240 | • Randomised, open-label, parallel-group, multi-centre trial<br>• Arm 1: camizestrant (75mg)<br>• Arm 2: camizestrant (150mg)<br>• Arm 3: camizestrant (300mg)<br>• Arm 4: *Faslodex* | • Primary endpoint: PFS | • FPCD: Q2 2020<br>• LPCD: Q3 2021<br>• Data readout: Q4 2022<br>• Primary endpoint met at 75mg and 150mg doses |
| **Phase II SERENA-3 NCT04588298** | HR+ HER2-negative early breast cancer | 135 | • Randomised, open-label, parallel-group, multi-centre trial<br>• camizestrant | • Primary endpoint: change in ER expression between pre- and on-treatment tumour biopsies | • FPCD: Q4 2020<br>• LPCD: Q2 2023<br>• Data readout: Q3 2023 |



As of 6 February 2025.
Appendix: Glossary.

# camizestrant (AZD9833, next-generation oral SERD)

## Breast cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT04541433** | HR+ HER2-negative advanced breast cancer | 18 | • Open-label trial<br>• camizestrant<br>• Japan only | • Primary endpoints: safety and tolerability<br>• Secondary endpoint: PK parameters | • FPCD: Q4 2020<br>• LPCD: Q1 2022<br>• Data readout: Q1 2023 |
| **Phase I**<br>**SERENA-1**<br>**NCT03616587** | HR+ HER2-negative advanced breast cancer | 396 | • Escalation phase: open-label multi-centre trial<br>• Cohort 1: camizestrant<br>• Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), *Truqap*, ribociclib (+/- anastrozole) or anastrozole<br>• Expansion phase: randomised expansion cohort(s)<br>• Cohort 1: camizestrant<br>• Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), *Truqap*, ribociclib (+/- anastrozole) or anastrozole | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and anti-tumour activity | • FPCD: Q4 2018<br>• LPCD: Q1 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT04818632** | HR+ HER2-negative metastatic breast cancer in Chinese patients | 30 | • Dose escalation: camizestrant<br>• Dose expansion:<br>• Cohort 1: camizestrant<br>• Cohort 2: camizestrant + palbociclib<br>• Cohort 3: camizestrant + everolimus<br>• China only | • Primary endpoints: safety, tolerability and PK parameters<br>• Secondary endpoint: anti-tumour activity | • FPCD: Q1 2021<br>• LPCD: Q1 2023<br>• Data readout: Q4 2023 |



As of 6 February 2025.
Appendix: Glossary.

# ceralasertib (AZD6738, ATR inhibitor)

## Multiple cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**LATIFY**<br>**NCT05450692** | Post-IO NSCLC | 594 | • Double-arm randomised<br>• Arm 1: ceralasertib + *Imfinzi*<br>• Arm 2: docetaxel | • Primary endpoint: OS<br>• Secondary endpoints: PFS, ORR, DoR, TTR, DCR, PFS2 and TTD | • FPCD: Q4 2022<br>• Data anticipated: H2 2025 |
| **Phase I/II**<br>**NCT02264678** | Solid tumours | 466 | • Module 1: ceralasertib + carboplatin<br>• Module 2: ceralasertib dose escalation, ceralasertib + *Lynparza*<br>• Module 3: ceralasertib + *Imfinzi*<br>• Module 4: ceralasertib monotherapy + *Lynparza* + *Imfinzi* (food effect/QT)<br>• Module 5: ceralasertib + saruparib<br>• Global trial – North America, Europe and South Korea | • Primary endpoints: safety and tolerability, efficacy and PK parameters | • FPCD: Q4 2014<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# rilvegostomig (AZD2936, PD-1/TIGIT bispecific mAb)

## Lung cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ARTEMIDE-Lung03**<br>**NCT06627647**<br>**Partnered (Compugen)** | Non-squamous NSCLC 1L patients whose tumours express PD-L1 (TC ≥1%) | 878 | • Randomised, double-blind, multi-centre trial<br>• Arm 1: rilvegostomig + platinum-based doublet chemotherapy followed by rilvegostomig monotherapy + pemetrexed in maintenance<br>• Arm 2: pembrolizumab + platinum-based doublet chemotherapy followed by pembrolizumab monotherapy + pemetrexed in maintenance | • Primary endpoints: PFS and OS<br>• Secondary endpoints: OS, ORR and DoR | • FPCD: Q4 2024<br>• Data anticipated: >2026 |
| **Phase III**<br>**ARTEMIDE-Lung02**<br>**NCT06692738**<br>**Partnered (Compugen)** | Squamous NSCLC 1L patients whose tumours express PD-L1 (TC ≥1%) | 880 | • Randomised, double-blind, multi-centre trial<br>• Arm 1: rilvegostomig + platinum-based doublet chemotherapy followed by rilvegostomig maintenance<br>• Arm 2: pembrolizumab + platinum-based doublet chemotherapy followed by pembrolizumab maintenance | • Primary endpoints: PFS and OS<br>• Secondary endpoint: OS, ORR and DoR | • FPCD: Q4 2024<br>• Data anticipated: >2026 |
| **Phase I/II**<br>**ARTEMIDE-01**<br>**NCT04995523**<br>**Partnered (Compugen)** | NSCLC | 199 | • Open-label, dose escalation and dose expansion trial<br>• Part A: dose escalation in CPI-experienced NSCLC patients with rilvegostomig i.v. monotherapy<br>• Part B: dose expansion in CPI-experienced NSCLC patients with rilvegostomig i.v. monotherapy<br>• Part C: dose expansion in CPI-naive NSCLC patients with rilvegostomig i.v. monotherapy<br>• Part D: randomised dose expansion in CPI-naive NSCLC patients with rilvegostomig i.v. monotherapy<br>• Part E: dose expansion in CPI-naive stage IV squamous NSCLC patients with rilvegostomig i.v. monotherapy<br>• Global trial | • Primary endpoints (Part A): safety, RP2D and MTD<br>• Primary endpoints (Part B): safety and efficacy (ORR)<br>• Primary endpoints (Part C): safety and efficacy (ORR)<br>• Primary endpoints (Part D): safety and efficacy (ORR)<br>• Primary endpoints (Part E): safety and efficacy (ORR)<br>• Secondary endpoints: PK parameters, PD (receptor occupancy), efficacy (DCR, DoR, DRR, PFS) | • FPCD: Q4 2021<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# rilvegostomig (AZD2936, PD-1/TIGIT bispecific mAb)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIb<br>GEMINI-Gastric<br>NCT05702229<br>Partnered (Compugen)** | Gastric cancer | 240 | • Open-label gastric platform trial<br>• Sub-study 1: volrustomig combined with XELOX or FOLFOX<br>• Sub-study 2: rilvegostomig combined with XELOX or FOLFOX<br>• Sub-study 3: AZD0901 combined with volrustomig plus fluorouracil or capecitabine<br>• Sub-study 4: AZD0901 combined with rilvegostomig plus fluorouracil or capecitabine<br>• Sub-study 5: AZD7789 combined with XELOX or FOLFOX<br>• Sub-study 6: AZD0901 combined with AZD7789 plus fluorouracil or capecitabine | • Primary endpoints: safety and efficacy (ORR and PFS6)<br>• Secondary endpoints: DoR, OS, PK, ADA and safety | • FPCD: Q1 2023<br>• Data anticipated: 2026 |
| **Phase IIb<br>GEMINI-Hepatobiliary<br>NCT05775159<br>Partnered (Compugen)** | HCC, BTC | 260 | • Open-label hepatobiliary platform trial<br>• HCC sub-study:<br>• Cohort 1A: volrustomig monotherapy<br>• Cohort 1B: volrustomig combination with bevacizumab<br>• Cohort 1C: volrustomig combination with lenvatinib<br>• Cohort 1D: volrustomig combination with rilvegostomig and bevacizumab<br>• Cohort 1E: rilvegostomig combination with bevacizumab<br>• BTC sub-study:<br>• Cohort 2A: rilvegostomig combination with gemcitabine and cisplatin<br>• Cohort 2B: volrustomig combination with gemcitabine and cisplatin | • Primary endpoints (HCC sub-study): safety and efficacy (ORR)<br>• Primary endpoints (BTC sub-study): safety and efficacy (PFS6)<br>• Secondary endpoints: DoR, OS, PK and ADA | • FPCD: Q2 2023<br>• Data anticipated: 2026 |
| **Phase III<br>ARTEMIDE-Biliary01<br>NCT06109779<br>Partnered (Compugen)** | BTC with curative intent | 750 | • Randomised, double-Blind, placebo-controlled, multicenter<br>• Arm 1: rilvegostomig + investigator's choice of chemotherapy (capecitabine, S-1 (tegafur/gimeracil/oteracil) or gemcitabine/cisplatin)<br>• Arm 2: placebo + investigator's choice of chemotherapy (capecitabine, S-1 (tegafur/gimeracil/oteracil) or gemcitabine/cisplatin) | • Primary endpoint: RFS<br>• Secondary endpoint: OS | • FPCD: Q4 2023<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# saruparib (AZD5305, PARP1 inhibitor)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**EvoPAR-Prostate01**<br>**NCT06120491** | HRRm and non-HRRm mCSPC | 1800 | • Randomised, placebo-controlled trial<br>• Arm 1: saruparib + physician's choice NHA (abiraterone, darolutamide or enzalutamide)<br>• Arm 2: placebo + physician's choice NHA (abiraterone, darolutamide or enzalutamide) | • Primary endpoint: rPFS<br>• Secondary endpoints: OS and PFS2 | • FPCD: Q4 2023<br>• Data anticipated: >2026 |
| **Phase III**<br>**EvoPAR-Breast01**<br>**NCT06380751** | *BRCA1*, *BRCA2*, or *PALB2*m, HR-positive, HER2-negative advanced breast cancer | 500 | • Randomised, open-label trial<br>• Arm 1: saruparib (AZD5305) + camizestrant<br>• Arm 2: physician's choice CDK4/6i + physician's choice ET<br>• Arm 3: physician's choice CDK4/6i + camizestrant | • Primary endpoint: PFS (BICR)<br>• Secondary endpoints: PFS2 and OS | • FPCD: Q3 2024<br>• Data anticipated: >2026 |
| **Phase I/IIa**<br>**PETRA**<br>**NCT04644068** | Advanced solid tumours | 804 | • Modular, open-label, multi-centre dose escalation and expansion trial<br>• Module 1: saruparib<br>• Module 2: saruparib + paclitaxel<br>• Module 3: saruparib + carboplatin +/- paclitaxel<br>• Module 4: saruparib + *Enhertu*<br>• Module 5: saruparib + *Datroway*<br>• Module 6: saruparib + camizestrant | • Primary endpoints: safety, tolerability and PK parameters<br>• Secondary endpoint: efficacy | • FPCD: Q4 2020<br>• Data anticipated: >2026 |
| **Phase I/IIa**<br>**PETRANHA**<br>**NCT05367440** | Metastatic prostate cancer | 190 | • Multi-arm, open-label, non-randomised, multi-centre trial of saruparib in combination with new hormonal agents in patients with metastatic prostate cancer<br>• Arm 1: saruparib + enzalutamide<br>• Arm 2: saruparib + abiraterone acetate<br>• Arm 3: saruparib + darolutamide<br>• Arm 4: saruparib + apalutamide | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and efficacy | • FPCD: Q2 2022<br>• Data anticipated: >2026 |
| **Phase I**<br>**NCT05573724** | Locally advanced, unresectable or metastatic solid tumours | 16 | • Part A: to assess the effect of multiple doses of itraconazole on the single-dose PK parameters of saruparib which will last up to 13 days and follows a non-randomised, open-label, 2 intervention design<br>• Part B: option to continue with saruparib monotherapy after completing Part A and whilst obtaining clinical benefit | • Primary endpoint: PK parameters<br>• Secondary endpoints: safety and tolerability | • FPCD: Q4 2022<br>• LPCD: Q2 2023<br>• Data readout: Q4 2023<br>• Primary endpoint met |
| **Phase I**<br>**ASCERTAIN**<br>**NCT05938270** | Newly diagnosed prostate cancer | 120 | • Open-label, randomised, multi-centre trial | • Primary endpoint: to assess the effects of treatment on γH2AX change<br>• Secondary endpoints: safety and tolerability, impact on surgical feasibility and change in Ki67 | • FPCD: Q3 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# volrustomig (MEDI5752, PD-1/CTLA-4 bispecific mAb)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase Ib**<br>**NCT04522323** | Advanced renal cell carcinoma | 67 | • Open-label, dose escalation and dose expansion trial<br>• Arm 1: volrustomig + axitinib<br>• Arm 2: volrustomig + lenvatanib | • Primary endpoints (escalation): safety, MTD, RP2D, tolerability and anti-tumour activity of combination (ORR)<br>• Secondary endpoints: PK parameters, ADA and anti-tumour activity (PFS, OR, DoR, DCR, TTR, OS) | • FPCD: Q3 2020<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT03530397** | Advanced solid tumours | 400 | • Open-label, dose escalation and dose expansion trial<br>• Dose escalation: volrustomig i.v.<br>• Dose expansion: volrustomig i.v. as monotherapy and + chemotherapy<br>• Arm 1: volrustomig i.v.<br>• Arm 2: volrustomig i.v., pemetrexed + carboplatin<br>• Arm 3: pembrolizumab, pemetrexed + carboplatin<br>• Arm 4: volrustomig i.v., taxane (paclitaxel or nab-paclitaxel) + carboplatin | • Primary endpoints (escalation): safety and tolerability, MTD, OBD and HPDD<br>• Primary endpoint (expansion): antitumour activity based on ORR<br>• Secondary endpoints: PK parameters, ADA, tumoural baseline PD-L1, anti-tumour activity (OR, DoR, DCR, PFS, OS) | • FPCD: Q2 2018<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

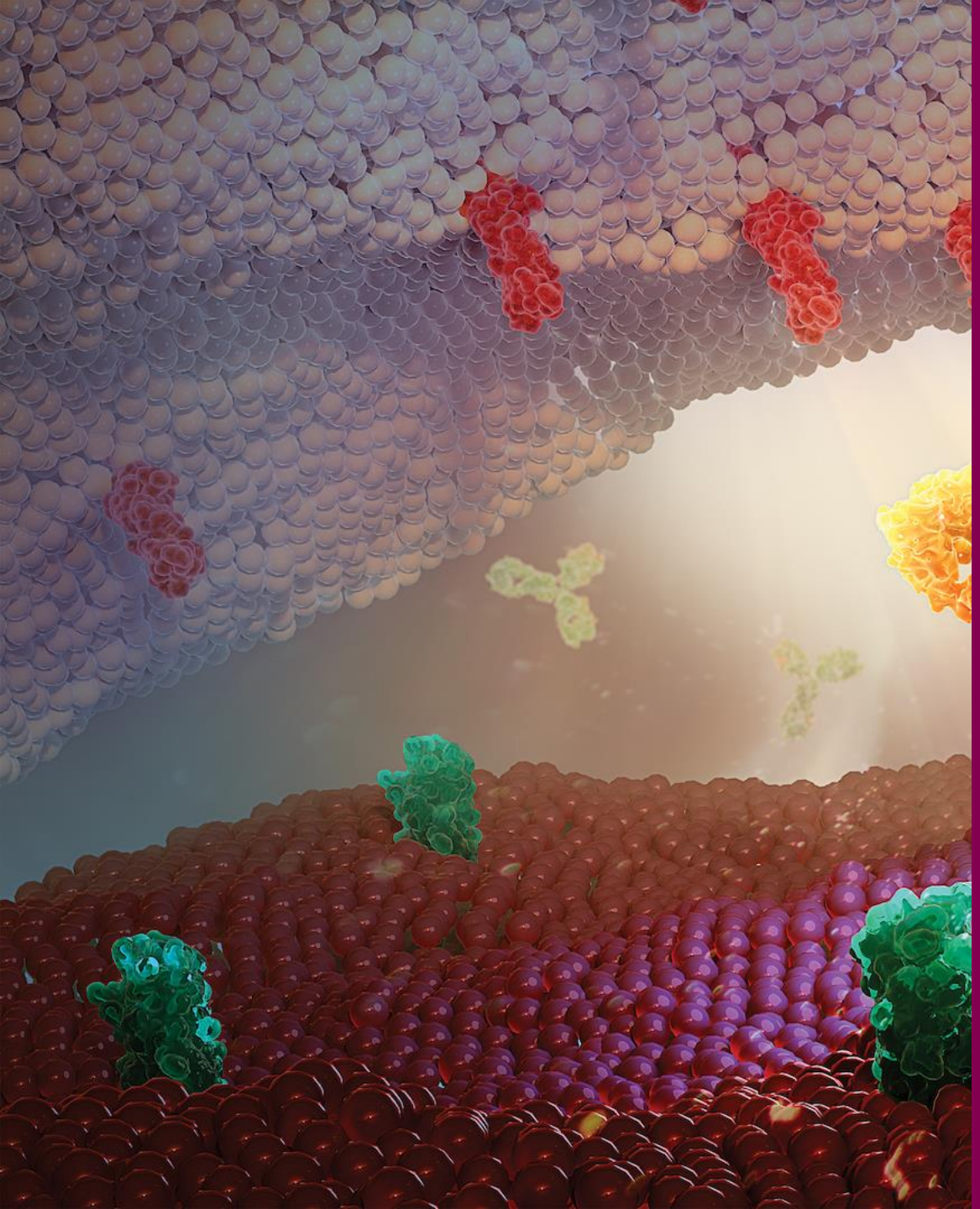
# volrustomig (MEDI5752, PD-1/CTLA-4 bispecific mAb)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>eVOLVE-Cervical<br>NCT06079671** | High-risk locally advanced cervical cancer with no progression following platinum-based cCRT | 800 | • Randomised, double-blind, placebo-controlled, multi-centre trial<br>• Arm 1: volrustomig<br>• Arm 2: placebo | • Primary endpoint: PFS (Inv, ITT)<br>• Secondary endpoints: OS, ORR, DoR | • FPCD: Q4 2023<br>• Data anticipated: >2026 |
| **Phase III<br>eVOLVE-Lung02<br>NCT05984277** | 1L mNSCLC with PD-L1 <50% | 900 | • Double-arm randomised, open-label trial<br>• Arm 1: volrustomig + chemotherapy<br>• Arm 2: pembrolizumab + chemotherapy | • Primary endpoints: OS and PFS (PD-L1 < 1%)<br>• Secondary endpoints: PFS (ITT), ORR and DoR<br>•<br>• | • FPCD: Q4 2023<br>• Data anticipated: >2026 |
| **Phase III<br>eVOLVE-Meso<br>NCT06097728** | 1L unresectable malignant pleural mesothelioma | 600 | • Double-arm, randomised, open-label trial<br>• Arm 1: volrustomig + chemotherapy<br>• Arm 2: chemotherapy or nivolumab + ipilimumab | • Primary endpoint: OS<br>• Secondary endpoints: PFS, landmark OS, landmark PFS and ORR | • FPCD: Q4 2023<br>• Data anticipated: >2026 |
| **Phase III<br>eVOLVE-HNSCC<br>NCT06129864** | Unresected, locally advanced HNSCC | 1145 | • Randomised, double-blind, placebo-controlled, multi-centre trial<br>• Arm 1: volrustomig<br>• Arm 2: observational | • Primary endpoint: PFS (BICR, PD-L1 expressing tumours)<br>• Secondary endpoints: PFS (BICR, ITT), landmark PFS, OS (PD-L1 expressing tumours), landmark OS and OS (ITT) | • FPCD: Q1 2024<br>• Data anticipated: >2026 |
| **Phase IIb<br>eVOLVE-01<br>NCT06448754** | NSCLC | 120 | • Platform, randomised, open-label, multicenter, global trial<br>• Arm 1A: volrustomig dose regimen 1 + chemotherapy<br>• Arm 1B: volrustomig dose regimen 2 + chemotherapy | • Primary endpoints: safety, & tolerability, ORR<br>• Secondary endpoints: DCR, DOR, PFS, OS | • FPCD: Q3 2024<br>• Data anticipated: H2 2025 |
| **Phase II<br>eVOLVE-02<br>NCT06535607** | Advanced/metastatic solid tumours | 60 | • Platform, multi-centre trial<br>• Sub-study 1: volrustomig monotherapy in participants with cervical cance<br>• Sub-study 2: volrustomig monotherapy in participants with head and neck squamous cell carcinoma | • Primary endpoints: ORR and safety<br>• Secondary endpoints: DOR, PFS, TTR, OS, PK parameters and immunogenicity | • FPCD: Q3 2024<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.



# Oncology:
## early-stage development

AstraZeneca

# AZD0022 (KRASG12D)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**ALAFOSS-01**<br>**NCT06599502** | Metastatic or locally advanced colorectal cancer, pancreatic ductal adenocarcinoma and NSCLC, with *KRASG12D* mutation | 430 | • Open-label, multi-centre trial with FIH modular protocol design<br>• Module 1: AZD0022 monotherapy with Part A (dose escalation) + Part B (dose optimisation) and Part C (potential dose expansion)<br>• Module 2: AZD0022 + cetuximab with Part A (dose escalation) + Part B (dose optimization) and Part C (potential dose expansion) | • Primary endpoints (Part A and B): safety, tolerability and determination of MTD and/or OBD of AZD0022 as a monotherapy or combination<br>• Primary endpoint (Part C): ORR<br>• Secondary endpoints (Part A and B): ORR, CR rate, DoR, DCR, DRR, TTR, PFS, OS, tumour markers, ctDNA and PK parameters<br>• Secondary endpoints (Part C): safety and tolerability, CR rate, DoR, DCR, DRR, TTR, PFS, OS, tumour markers, ctDNA and PK parameters | • FPCD: Q4 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD0120 (GC012F, autologous anti-CD19 and anti-BCMA CAR-T)

## Blood cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**NCT05850234** | Relapsed/refractory multiple myeloma | 68 | • Open-label, single-arm, multi-centre trial | • Primary endpoints: incidence of AEs, DLTs and ORR<br>• Secondary endpoints: DOR, PFS, OS and PK parameters | • FPCD: Q3 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD0171 (anti-LIF mAb)

## Cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**NCT04999969** | 1L metastatic pancreatic ductal adenocarcinoma | 126 | • Open-label, non-randomised trial<br>• AZD0171 + *Imfinzi* + gemcitabine, nab-paclitaxel | • Primary endpoints: safety and OS at 12 months<br>• Secondary endpoints: ORR, DoR and PFS | • FPCD: Q1 2022<br>• LPCD: Q4 2023<br>• Data readout: Q4 2024<br>• Trial discontinued due to efficacy |



As of 6 February 2025.
Appendix: Glossary.

# AZD0305 (GPRC5D ADC)

## Blood cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**NCT06106945** | r/r multiple myeloma | 84 | • Open-label, dose escalation and dose expansion trial<br>• Phase I: AZD0305 prescribed at specified dose levels<br>• Phase II: AZD0305 prescribed as RP2D | • Primary endpoints: occurrence of dose-limiting toxicities and incidence and severity of AEs and SAEs<br>• Secondary endpoints: ORR, DoR, PFS, OS, PK parameters and immunogenicity | • FPCD: Q4 2023<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# AZD0754 (STEAP2 dnTGFβRII-armoured CAR-T)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**APOLLO**<br>**NCT06267729** | Metastatic castration resistance prostate cancer with prior NHA and taxane exposure | 60 | • Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components | • Primary endpoints (Phase I): DLT, AEs (including AESI and SAEs), determination of recommended dose for expansion phase<br>• Secondary endpoints (Phase I): PSA related changes (PSA50, PSA90), radiological assesssment according to RECIST v1.1 and PCWG3 (ORR, BOR, DRR, DCR, TTR, rPFS, OS), PK parameters (Cmax, Tmax, Tlast, AUC) | • FPCD: Q2 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD1390 (ATM inhibitor)

## Cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT03423628** | Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients | 165 | • Open-label trial<br>• Arm 1: recurrent GBM, AZD1390 + RT in dose escalation cohorts (Japan safety/PK cohorts added); optional food effect cohort initiated<br>• Arm 3: primary GBM, AZD1390 + RT in dose escalation cohorts | • Primary endpoints: safety, tolerability and MTD<br>• Secondary endpoints: PK parameters and preliminary assessment of anti-tumour activity | • FPCD: Q2 2018<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD2068 (FPI-2068, EGFR cMET radioconjugate)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06147037** | Advanced solid tumours | 110 | • Multicentre, open-label dose escalation trial<br>• Part A: optimisation of FPI-2053 dose (treatment with dose level 1 of [225Ac]-AZD2068 - fixed dose)<br>• Part B: dose escalation of [225Ac]-AZD2068 with optimal FPI-2053 | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: anti-tumour activity, immunogenicity and PK parameters | • FPCD: Q3 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD3470 (PRMT5)

## Solid tumours and blood cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**PRIMROSE**<br>**NCT06130553** | MTAP-deficient advanced solid tumours | 210 | • Arm 1: AZD3470<br>• Global trial – 8 countries | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and clinical efficacy | • FPCD: Q1 2024<br>• Data anticipated: H2 2025 |
| **Phase I**<br>**PRIMAVERA**<br>**NCT06137144** | R/R haematologic malignancies | 110 | • Modular Phase I/II open-label dose escalation and expansion trial<br>• Module 1 – Part A (dose escalation): AZD3470 monotherapy<br>• Module 1 – Part B (dose expansion/optimisation): AZD3470 monotherapy | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and clinical effacacy | • FPCD: Q1 2024<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD5335 (anti-FRα TOP1i ADC)

## Solid tumours, ovarian cancer, lung cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**FONTANA**<br>**NCT05797168** | Advanced solid tumour malignancies | 446 | • Module 1: AZD5335 monotherapy<br>• Module 2: AZD5335 in combination with saruparib<br>• Module 3: AZD5335 in combination with bevacizumab<br>• Module 4: AZD5335 in combination with carboplatin +/- bevacizumab | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: efficacy and PK parameters | • FPCD: Q3 2023<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD5492 (CD20 TITAN TCE)

## Blood cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**TITANIUM**<br>**NCT06542250** | CLL, MCL, LBCL, FL | 176 | • Module 1: AZD5492 monotherapy<br>• AZD5492 monotherapy for r/r B-cell malignancies | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: preliminary efficacy (ORR, CRR, DoR, PFS, OS), PK parameters and immunogenicity | • FPCD: Q3 2024<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD5851 (GPC3 dnTGFβRII-armoured CAR-T)

## Gastrointestinal cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**ATHENA**<br>**NCT06084884** | GPC3-positive advanced/recurrent HCC | 93 | • Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components<br>• AZD5851 | • Primary endpoints (Phase I): DLT, AEs (including AESI and SAEs and determination of recommended dose for expansion phase<br>• Secondary endpoints (Phase I): ORR per RECIST v. 1.1, TTR, DCR, DRR, BoR, DoR, PFS, OS and PK parameters (Cmax, Tmax, Tlast, AUC) | • FPCD: Q1 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD5863 (CLDN18.2 CD3 bispecific antibody)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06005493** | Advanced or metastatic solid tumours with CLDN18.2 expression | 200 | • Part A: dose escalation phase to determine the safety, tolerability, RP2D, and/or MTD of AZD5863<br>• Part B: dose expansion phase to further characterise the safety profile and evaluate anti-tumour activity of AZD5863 | • Primary endpoints (Part A): safety and tolerability<br>• Primary endpoints (Part B): safety, tolerability and preliminary anti-tumour activity<br>• Secondary endpoints: preliminary anti-cancer activity, PK parameters and immunogenicity | • FPCD: Q4 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD6422 (CLDN18.2 CAR-T)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT05981235** | Advanced or metastatic CLDN18.2-positive GI tumours | 96 | • Open-label trial, dose escalation (Part 1) and dose expansion (Part 2) | • Primary endpoints: incidence of TEAEs, AESIs and SAEs, DLT and changes from baseline in vital signs, laboratory parameters, physical examination and 12-lead ECG<br>• Secondary endpoints: ORR, DoR, DCR and PFS | • FPCD: Q4 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD7003 (GPC3 CAR-T)

## Hepatocellular carcinoma (HCC)

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II<br>STARLIGHT<br>NCT06590246** | GPC3-positive advanced/recurrent HCC | 121 | • Open-label, single-arm, multi-centre trial with dose escalation and dose expansion components | • Primary endpoints (Phase I): DLT, AEs (including AESI and SAEs), determination of recommended dose for expansion phase<br>• Secondary endpoints (Phase I): ORR per RECIST v. 1.1, TTR, DCR, DRR, BoR, DoR, PFS and OS; PK parameters (Cmax, Tmax, Tlast, AUC) | • FPCD: Q4 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# puxitatug samrotecan (AZD8205, B7H4 ADC)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**NCT05123482** | Breast cancer, BTC, ovarian cancer, endometrial cancer | 340 | • Open-label dose escalation and expansion trial<br>• Sub-study 1: puxitatug samrotecan monotherapy<br>• Sub-study 2: puxitatug samrotecan + rilvegostomig | • Primary endpoints: AE, SAE, DLTs, changes in lab and preliminary efficacy parameters<br>• Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters and ADA | • FPCD: Q1 2022<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD8421 (CDK2 inhibitor)

## Breast cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**CYCAD-1**<br>**NCT06188520** | ER+ HER2-negative advanced breast cancer | 204 | • Module 1: AZD8421<br>• Module 2: AZD8421+ camizestrant + one or more of abemaciclib or ribociclib or palbociclib<br>• Global trial – 5 countries | • Primary endpoints: safety and tolerablility<br>• Secondary endpoints: PK parameters | • FPCD: Q4 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD9574 (PARP1-sel BBB inhibitor)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/IIa**<br>**CERTIS-1**<br>**NCT05417594** | Advanced solid malignancies | 490 | • Modular, open-label, multi-centre dose escalation and expansion trial<br>• Module 1: AZD9574 monotherapy<br>• Module 2: AZD9574 + temozolomide<br>• Module 3: [11C]AZ14193391 + AZD9574 or [11C]AZ14193391 + AZD9574 + temozolomide<br>• Module 4: AZD9574 + *Enhertu*<br>• Module 5: AZD9574 + *Datroway* | • Primary endpoints: safety and tolerability of AZD9574 as monotherapy and in combination with anti-cancer agents, determination of PARP1 occupancy in brain by AZD9574 at examined doses and plasma concentration and evaluation of safety of radioligand [11C]AZ14193391<br>• Secondary endpoints: PK parameters and efficacy of AZD9574 as monotherapy and in combination with anti-cancer agents | • FPCD: Q3 2022<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# AZD9592 (EGFR-cMET TOP1i ADC)

## Lung cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**EGRET**<br>**NCT05647122** | Advanced solid tumours including NSCLC, HNSCC and CRC | 162 | • Escalation phase, open-label, multi-centre trial<br>• Arm 1: AZD9592<br>• Arm 2: AZD9592 + *Tagrisso*<br>• Arm 3: AZD9592 + 5FU + bevacizumab | • Primary endpoints (escalation): safety and tolerability<br>• Primary endpoints (expansion): safety, tolerability and anti-tumour activity<br>• Secondary endpoints (escalation): PK parameters, immunogenicity and anti-tumour activity<br>• Secondary endpoints (expansion): PK parameters and immunogenicity | • FPCD: Q1 2023<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# AZD9829 (CD123 TOP1i ADC)

## Blood cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**ADC123**<br>**NCT06179511** | CD123-positive haematological malignancies | 104 | • Open-label, multi-centre trial<br>• Module 1: dose escalation with ascending dose level cohorts of AZD9829 in AML and MDS participants | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and efficacy | • FPCD: Q1 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# FPI-2265 (PSMA radioconjugate)

## Prostate cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**AlphaBreak**<br>**NCT06402331**<br>**Partnered (Fusion)** | PSMA-positive mCRPC previously treated with lutetium-PSMA therapy | 60 | • Open-label, randomised, multi-centre trial<br>• 3 arm dose optimisation<br>• Arm A: FPI-2265, IV Q4W<br>• Arm B: FPI-2265, IV Q6W<br>• Arm C: FPI-2265, IV Q8W | • Primary endpoints: PSA50 and safety | • FPCD: Q2 2024<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# IPH5201 (CD39 mAb)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT04261075**<br>**Partnered (Innate Pharma)** | Advanced solid tumours | 57 | • Open-label, dose escalation trial to determine MTD of IPH5201 as monotherapy, or in combination with *Imfinzi* +/- oleclumab<br>• Part 1: IPH5201 monotherapy dose escalation to MTD<br>• Part 2: IPH5201 + *Imfinzi* dose escalation to MTD<br>• Part 3: IPH5201 + *Imfinzi* + oleclumab dose escalation to MTD<br>• Route of administration: i.v.<br>• Global trial – US and EU | • Primary endpoints: AE, SAE and DLT<br>• Secondary endpoints: OR, DC, PK parameters and ADA | • FPCD: Q1 2020<br>• LPCD: Q2 2022<br>• Data readout: Q2 2023 |



As of 6 February 2025.
Appendix: Glossary.

# NT-112 (KRAS G12D specific TCR)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06218914** | Unresectable, advanced and/or metastatic non-small cell lung cancer, colorectal adenocarcinoma, pancreatic adenocarcinoma, endometrial cancer or any solid tumour histology positive for *KRAS G12D* mutation | 24 | • Open-label, single-arm, multi-centre trial with dose escalation | • Primary endpoints: incidence of DLTs, TEAEs and SAEs<br>• Secondary endpoints: ORR per RECIST v.1.1, BOR, DOR, CBR (CR, PR, SD), TTR, PFS and OS | • FPCD: Q1 2024<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# NT-125 (autologous, multi-specific neoantigen-targeting TCR-T)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**EudraCT: 2021-006406-73** | Adults with recurrent or metastatic NSCLC, melanoma, colorectal adenocarcinoma, HNSCC, bladder carcinoma, TNBC, cervical squamous cell carcinoma and adenocarcinoma or microsatellite instability-high/mismatch repair-deficient solid tumours | 42 | • Open-label, single-arm, single-centre trial with dose escalation and dose expansion components<br>• Arm 1: NT-125 | • Primary endpoint (Phase Ia): incidence of AEs defined as DLTs<br>• Primary endpoint (Phase Ib): ORR per RECIST v.1.1<br>• Secondary endpoints (Phase Ia): percentage of pre-screened and enrolled subjects that receive treatment<br>• Secondary endpoints (Phase Ib): percentage change tumour size, best percentage change tumour size, DoR, clinical benefit rate, TTP, PFS and OS | • FPCD: Q2 2023<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# NT-175 (TP53-armored TCR)

## Multiple cancers

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT05877599** | Unresectable, advanced, and/or metastatic solid tumours positive for HLA-A*02:01 and the TP53 R175H mutation | 24 | • Open-label, single-arm, multi-centre trial with dose escalation | • Primary endpoint: incidence of DLTs, TEAEs and SAEs<br>• Secondary endpoints: ORR per RECIST v.1.1, BOR, DOR, CBR (CR, PR, SD), TTR, PFS and OS | • FPCD: Q3 2023<br>• Data anticipated: H1 2025 |



As of 6 February 2025.
Appendix: Glossary.

# oleclumab (CD73 mAb)

## Solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase Ib/II**<br>**NCT03611556** | Pancreatic 1L and 2L with prior gemcitabine-based chemotherapy | 339 | • Arm A1: gemcitabine and nab paclitaxel i.v.<br>• Arm A2: gemcitabine and nab paclitaxel i.v. + oleclumab i.v.<br>• Arm A3: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. + *Imfinzi* i.v.<br>• Arm B1: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v.<br>• Arm B2: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v.<br>• Arm B3: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. + *Imfinzi* i.v.<br>• Global trial – US, Norway, Spain and Australia | • Primary endpoints: safety and anti-tumour activity<br>• Secondary endpoints: PFS, PK parameters, immunogenicity, safety and anti-tumour activity | • FPCD: Q2 2018<br>• LPCD: Q3 2022<br>• Data readout: Q2 2023 |



As of 6 February 2025.
Appendix: Glossary.

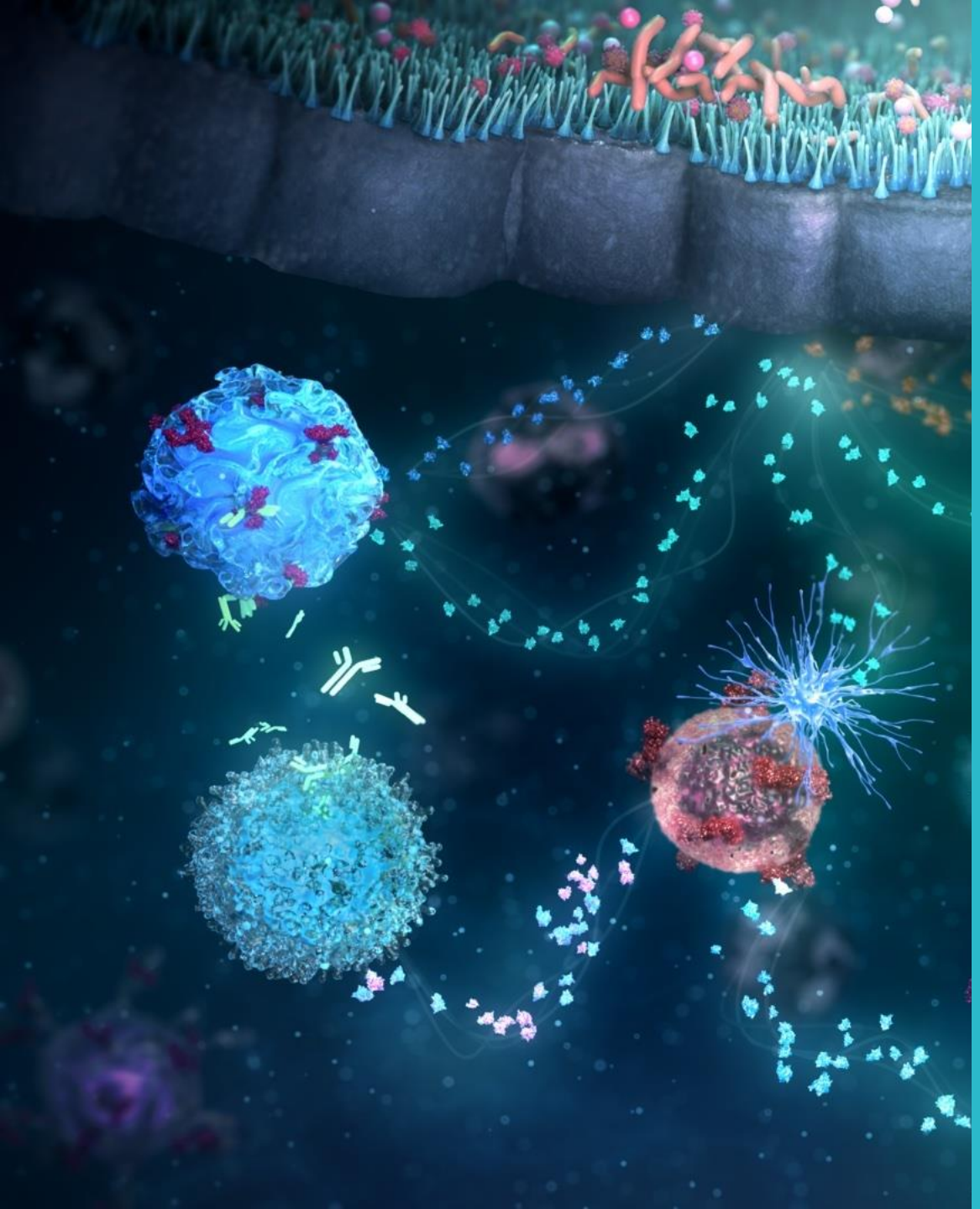
# sabestomig (AZD7789, PD-1/TIM3 bispecific mAb)

## Cancer

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/IIa**<br>**NCT04931654** | NSCLC, gastric cancer and other tumours | 192 | • Open-label, non-randomised dose escalation and dose expansion trial<br>• Part A: dose escalation in post-IO NSCLC patients with sabestomig i.v. monotherapy<br>• Part B: dose expansion in post-IO and IO-naïve NSCLC patients and also post-IO gastric patients with sabestomig i.v. monotherapy<br>• Global trial | • Primary endpoints: AE, SAE, DLTs and ORR<br>• Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters, ADA and ctDNA | • FPCD: Q4 2021<br>• LPCD: Q2 2024<br>• Data anticipated: H1 2025<br>• Trial discontinued due to strategic portfolio prioritisation |



As of 6 February 2025.
Appendix: Glossary.



# BioPharmaceuticals: approved medicines and late-stage development

AstraZeneca

# *Wainua* (eplontersen, ligand-conjugated antisense)

## ATTR

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III<br>CARDIO-TTRansform<br>NCT04136171<br>Partnered (Ionis Pharmaceuticals, Inc.)** | Hereditary or wild-type transthyretin-mediated amyloid cardiomyopathy (ATTR-CM) | 1438 | • Arm 1: *Wainua* s.c.<br>• Arm 2: placebo | • Primary endpoints: composite outcome of CV mortality and recurrent CV clinical events at Week 140<br>• Secondary endpoints: 6MWT, KCCQ, CV events and CV mortality | • FPCD: Q1 2020<br>• Data anticipated: 2026 |
| **Phase III<br>NEURO-TTRansform<br>NCT04136184<br>Partnered (Ionis Pharmaceuticals, Inc.)** | Hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN) | 168 | • Arm 1: *Wainua* s.c.<br>• Arm 2: inotersen s.c. | • Primary endpoints (at Week 35): change from baseline in mNIS+7 and percent change from baseline in TTR concentration<br>• Secondary endpoint (Week 35): changes from baseline in Norfolk QOL<br>• Primary endpoints (at Week 66): change from baseline in mNIS+7, change from baseline in the Norfolk QoL-DN Questionnaire and percent change from baseline in TTR concentration | • FPCD: Q1 2020<br>• LPCD: Q3 2023<br>• Data readout: Q2 2022<br>• Co-primary endpoints met at Week 35 and Week 66 |
| **Phase III<br>EPIC-ATTR<br>NCT06194825** | ATTR-CM | 64 | • Arm 1: *Wainua* s.c. Q4W<br>• Arm 2: placebo<br>• China only | • Primary endpoint (at week 24): percent change from baseline in serum TTR concentration<br>• Secondary endpoints: PK, immunogenicity, disease biomarkers (NT pro-BNP, hsTnT) | • FPCD: Q4 2023<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# balcinrenone/dapagliflozin (MR modulator + SGLT2 inhibitor)

## Heart failure, CKD

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**BalanceD-HF**<br>**NCT06307652** | Heart failure patients with renal impairment (eGFR 20-60 ml/min) with heart failure event within the last 6 months | 4800 | • Randomised, double-blind, parallel-group, double-dummy, active-controlled, event-driven trial<br>• Arm 1: balcinrenone/dapagliflozin 15mg/10mg<br>• Arm 2: balcinrenone/dapagliflozin 40mg/10mg<br>• Arm 3: dapagliflozin 10mg | • Primary endpoints: time to first occurrences of any the components of the composite of CV death, HF hospitalisation and HF event without hospitalisation<br>• Secondary endpoints: total occurrences (first and recurrent) of the components of the composite of CV death, HF hospitalisation and HF event without hospitalization; time to CV death; the hierarchical composite endpoint of death from any cause, total HF events, and change from baseline in KCCQ total symptom score to 24-week post-randomisation; and time do death from any cause | • FPCD: Q2 2024<br>• Data anticipated: >2026 |
| **Phase IIb**<br>**MIRACLE**<br>**NCT04595370** | Heart failure with chronic kidney disease | 500 | • Randomised, stratified according to T2DM and eGFR (≥20 to <30 mL/min / ≥30 to <45 mL/min / ≥45 mL/min) for 12 weeks<br>• Arm 1: AZD9977 A + dapagliflozin 10mg<br>• Arm 2: AZD9977 B + dapagliflozin 10mg<br>• Arm 3: AZD9977 C + dapagliflozin 10mg<br>• Arm 4: dapagliflozin 10mg<br>• 12 weeks<br>• Global trial – 19 countries | • Primary endpoint: percent change from baseline in UACR at 12 weeks<br>• Secondary endpoints: percent change from baseline in UACR at 12 weeks to assess dose-response relationship; dose-response relationship of dapagliflozin and 3 doses of AZD9977 combined with dapagliflozin on UACR; safety, tolerability and serum potassium values; eGFR | • FPCD: Q2 2021<br>• LPCD: Q3 2023<br>• Data readout: Q4 2023 |
| **Phase IIb**<br>**MIRO-CKD**<br>**NCT06350123** | CKD | 300 | • Multicentre, randomised, double-blind, dose-finding, parallel group, double-dummy trial<br>• Arm 1: balcinrenone/dapagliflozin 15 mg/10 mg once daily<br>• Arm 2: balcinrenone/dapagliflozin 40 mg/10 mg once daily<br>• Arm 3: dapagliflozin 10 mg once daily | • Primary endpoint: Relative change in UACR from baseline to Week 12 | • FPCD: Q2 2024<br>• LPCD: Q4 2024<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# baxdrostat (selective aldosterone synthase inhibitor)

## Hypertension

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**BaxHTN**<br>**NCT06034743** | Patients with uncontrolled hypertension on two or more antihypertensive medications including patients with resistant hypertension | 720 | • Arm 1: baxdrostat 1mg QD<br>• Arm 2: baxdrostat 2mg QD<br>• Arm 3: placebo QD<br>• Global trial – 29 countries | • Primary endpoint: effect of baxdrostat vs. placebo on seated systolic blood pressure at Week 12<br>• Secondary endpoints: effect of baxdrostat vs. placebo on seated systolic blood pressure at 8 weeks after randomised withdrawal, safety and tolerability | • FPCD: Q1 2024<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**Bax24**<br>**NCT06168409** | Patients with resistant hypertension on three or more antihypertensive medications | 212 | • Arm 1: baxdrostat 2mg QD<br>• Arm 2: placebo QD<br>• Global trial – 29 countries | • Primary endpoint: effect of baxdrostat vs. placebo on ambulatory 24-hour average systolic blood pressure at Week 12 | • FPCD: Q2 2024<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**BaxAsia**<br>**NCT06344104** | Patients with uncontrolled hypertension on two or more antihypertensive medications including patients with resistant hypertension | 300 | • Arm 1: baxdrostat 1mg QD<br>• Arm 2 baxdrostat 2mg QD<br>• Arm 3: placebo QD<br>• Global Trial – 11 countries | • Primary endpoint: effect of baxdrostat vs. placebo on seated systolic blood pressure at Week 12<br>• Secondary endpoints: effect of baxdrostat vs. placebo on ambulatory 24-hour average systolic blood pressure, safety and tolerability | • FPCD: Q2 2024<br>• Data anticipated: 2026 |
| **Phase II**<br>**SPARK**<br>**NCT04605549** | Patients with primary aldosteronism | 18 | • Arm 1: baxdrostat 2-8mg QD<br>• US only | • Primary endpoints: safety and tolerability in patients with PA at doses from 2 to 8mg per day for 12 weeks and the reduction in SBP patients with PA after 12 weeks<br>• Secondary endpoints: reduction in DBP as a function of dose in patients with PA after 12 weeks of treatment, change in serum potassium and requirement for potassium supplementation and change in serum sodium and requirement for fluid or mineral replacement | • FPCD: Q3 2022<br>• LPCD: Q4 2024<br>• Data readout: Q1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# baxdrostat (selective aldosterone synthase inhibitor)

## Hypertension

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**HALO-OLE**<br>**NCT05459688** | Patients with uncontrolled hypertension who have completed CIN-107-124 | 175 | • Arm 1: baxdrostat 2mg QD<br>• US only | • Primary endpoints: safety and tolerability | • FPCD: Q2 2022<br>• LPCD: Q4 2023<br>• Data readout: Q2 2024 |
| **Phase II**<br>**FigHTN**<br>**NCT05432167** | Patients with uncontrolled hypertension and CKD | 194 | • Arm 1: baxdrostat (low dose)<br>• Arm 2: baxdrostat (high dose)<br>• Arm 3: placebo<br>• US only | • Primary endpoint: change from baseline in mean seated systolic blood pressure vs. placebo at Week 26<br>• Secondary endpoint: to evaluate the treatment effect on SBP at Week 26 by dosing strategy | • FPCD: Q2 2022<br>• LPCD: Q2 2024<br>• Data readout: Q3 2024 |
| **Phase II**<br>**NCT06336356** | Patients with uncontrolled hypertension on one or more antihypertensive medications | 45 | • Arm 1: baxdrostat 2mg QD<br>• Arm 2: placebo | • Primary endpoint: individual cortisol level before and after ACTH stimulation test at baseline and Week 8 | • FPCD: Q2 2024<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT06194032** | Healthy volunteers | 28 | • Arm 1: baxdrostat 16mg (single dose)<br>• Arm 2: baxdrostat 32mg (single dose)<br>• Arm 3: placebo (single dose)<br>• Arm 4: moxifloxacin 400mg (single dose) | • Primary endpoint: placebo-corrected change from baseline QTcF | • FPCD: Q1 2024<br>• LPCD: Q2 2024<br>• Data readout: Q3 2024 |
| **Phase I**<br>**NCT06357520** | Healthy volunteers | 14 | • Arm 1: baxdrostat 2mg and itraconazole 200mg<br>• US only | • Primary endpoint: AUCinf and Cmax | • FPCD: Q2 2024<br>• LPCD: Q2 2024<br>• Data readout: Q3 2024 |
| **Phase I**<br>**NCT06657105** | Healthy volunteers | 22 | • Arm1: baxdrostat 2mg and ethiny estradiol/levonorgestrel 0.06/0.3mg | • Primary endpoints: AUCinf, AUClast and Cmax | • FPCD: Q4 2024<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# baxdrostat/dapagliflozin (selective ASI/SGLT2)

## CKD

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**BaxDuo-Arctic**<br>**NCT06268873** | CKD and high blood pressure | 2500 | • Arm 1: baxdrostat/dapagliflozin QD<br>• Arm 2: dapagliflozin/placebo QD | • Primary endpoint: change from baseline in eGFR to post-treatment<br>• Secondary endpoints: change from baseline in SBP and UACR, kidney HCE and eGFR | • FPCD: Q2 2024<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2 inhibitor)

## Chronic kidney disease

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ZENITH High Proteinuria**<br>**NCT06087835** | CKD and high proteinuria | 1835 | • Randomised, parallel, multi-centre, double-blind trial<br>• Arm 1: zibotentan/dapagliflozin dose A or dose B<br>• Arm 2: dapagliflozin<br>• Global trial | • Primary endpoint: change in eGFR from baseline<br>• Secondary endpoints: change in UPCR from baseline to each participant's mean level; change in UACR from baseline to each participant's mean level; time to the first occurrence of any of the components of the renal composite endpoint of 40% sustained decline in eGFR or ESKD or renal death | • FPCD: Q4 2023<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# zibotentan/dapagliflozin (ETA receptor antagonist/SGLT2 inhibitor)

## Liver cirrhosis

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**ZEAL**<br>**NCT05516498** | Part A: participants with Child-Pugh A cirrhosis with features of portal hypertension and with no history of decompensation events<br>Part B: participants with a broader range of Child-Pugh A and Child-Pugh B cirrhosis with more severe disease | 195 | • Phase IIa/b multi-centre, randomised, double-blind, placebo-controlled, parallel group dose-ranging trial<br>• Part A - Arm 1: placebo<br>• Part A - Arm 2: zibotentan dose B + dapagliflozin<br>• Part B - Arm 1: placebo<br>• Part B - Arm 2: placebo + dapagliflozin<br>• Part B - Arm 3: zibotentan dose A + dapagliflozin<br>• Part B - Arm 4: zibotentan dose B + dapagliflozin<br>• Part B - Arm 5: zibotentan dose C + dapagliflozin<br>• Global trial | • Primary endpoint (Part A): absolute change in HVPG from baseline to Week 6 comparing zibotentan and dapagliflozin in combination vs. placebo<br>• Primary endpoint (Part B): absolute change in HVPG from baseline to Week 6 comparing zibotentan and dapagliflozin in combination and dapagliflozin mono vs. placebo | • FPCD: Q4 2022<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# *Airsupra* (PT027, SABA/ICS, pMDI)

## Asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIIb**<br>**BATURA**<br>**NCT05505734**<br>**Managed by Avillion (Avillion)** | Adults and adolescents with mild asthma | 2517 | • Randomised, double-blind, multi-centre, parallel-group, decentralised<br>• 12 to 52-week treatment period<br>• Arm 1: *Airsupra* MDI 160/180µg<br>• Arm 2: AS MDI 180µg<br>• US only | • Primary endpoint: time to first severe asthma exacerbation | • FPCD: Q3 2022<br>• LPCD: Q1 2024<br>• Data readout: Q4 2024<br>• Primary endpoint met |
| **Phase IIIb**<br>**ACADIA**<br>**NCT06307665** | Adolescents with asthma | 440 | • Randomised, double-blind, multi-center, parallel-group<br>• Arm 1: BDA MDI 160/180µg prn<br>• Arm 2: AS MDI 180µg prn<br>• Global trial | • Primary endpoint: severe asthma exacerbation rate (annualised)<br>• Secondary endpoints: time to first severe exacerbation, annualised total systemic corticosteroid exposure, safety (AEs and SAEs), PK sub-study (including Cmax, AUClast and AUCinf) | • FPCD: Q2 2024<br>• Data anticipated: >2026 |
| **Phase III**<br>**BAIYUN**<br>**NCT06471257** | Adult patients with asthma | 790 | • Randomised, double-blind, multi-centre, event-driven, parallel-group<br>• Arm 1: BDA MDI 160/180µg prn<br>• Arm 2: AS MDI 180 µg prn<br>• China only | • Primary endpoint: time to first severe exacerbation<br>• Secondary endpoints: Severe exacerbation rate (annualised), total systemic corticosteroid exposure, ACQ-5 responder, AQLQ+12 responder | • FPCD: Q3 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**PUTUO**<br>**NCT06514157** | Healthy volunteers | 14 | • Open-label, single-dose, single-centre trial<br>• BDA MDI 160µg/180µg (single dose) | • Primary endpoints: PK parameters for budesonide and albuterol include AUClast, AUCinf, Cmax, tmax, tlast, t½λz, CL/F and Vz/F | • FPCD: Q3 2024<br>• LPCD: Q3 2024<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# *Breztri*, Trixeo (LAMA/LABA/ICS)

## Asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**KALOS**<br>**NCT04609878** | Uncontrolled asthma | 2266 | • Randomised, double-blind, double-dummy, parallel group and multi-centre trial<br>• Treatments (24- to 52-week variable length)<br>• Arm 1: BGF 320/28.8/9.6µg BID MDI<br>• Arm 2: BGF 320/14.4/9.6µg BID MDI<br>• Arm 3: *Symbicort* Aerosphere 320/9.6µg BID MDI<br>• Arm 4: *Symbicort* 320/9µg BID pMDI<br>• Global trial | • Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24<br>• Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24 | • FPCD: Q1 2021<br>• Data anticipated: H1 2025 |
| **Phase III**<br>**LOGOS**<br>**NCT04609904** | Uncontrolled asthma | 2182 | • Randomised, double-blind, double dummy, parallel group and multi-centre trial<br>• Treatments (24- to 52-week variable length)<br>• Arm 1: BGF 320/28.8/9.6µg BID MDI<br>• Arm 2: BGF 320/14.4/9.6µg BID MDI<br>• Arm 3: *Symbicort* Aerosphere 320/9.6µg BID MDI<br>• Arm 4: *Symbicort* 320/9µg BID pMDI<br>• Global trial | • Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24<br>• Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24 | • FPCD: Q1 2021<br>• Data anticipated: H1 2025 |
| **Phase III**<br>**VATHOS**<br>**NCT05202262** | Inadequately controlled asthma despite treatment with medium dose ICS or ICS/LABA | 645 | • Randomised, double-blind, parallel group, multi-centre trial<br>• Treatments (24-week)<br>• Arm 1: *Symbicort* Aerosphere 320/9.6µg BID MDI<br>• Arm 2: PT009 160/9.6µg BID MDI<br>• Arm 3: BD 320µg BID MDI<br>• Arm 4:open-label *Symbicort* Turbuhaler 320/9µg BID<br>• Global trial | • Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 | • FPCD: Q1 2022<br>• Data anticipated: H1 2025 |
| **Phase III**<br>**LITHOS**<br>**NCT05755906** | Inadequately controlled asthma despite treatment with low dose ICS or ICS/LABA | 373 | • Randomised, double-blind, parallel group and multi-centre<br>• Treatments (12-week)<br>• Arm 1: PT009 160/9.6µg BID MDI<br>• Arm 2: BD 160µg BID MDI<br>• Global trial | • Primary endpoint: Change from baseline in forced expiratory volume in 1 second (FEV1) area under the curve 0 to 3 hours (AUC0-3) at Week 12 | • FPCD: Q1 2023<br>• Data anticipated: H1 2025 |



As of 6 February 2025.
Appendix: Glossary.

# *Breztri*, Trixeo (LAMA/LABA/ICS)

## COPD

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ATHLOS**<br>**NCT06067828** | COPD | 180 | • Randomised, double-blind, three-treatment, three-period, crossover trial<br>• Treatments (2-week treatment periods, 2-week washout between treatments)<br>• Arm 1: *Breztri* 320/14.4/9.6µg BID MDI<br>• Arm 2: *Symbicort* Aerosphere 320/9.6µg BID MDI<br>• Arm 3: placebo BID MDI | • Primary endpoint: change from baseline in isotime IC<br>• Secondary endpoint: change from baseline in constant work rate cycle ergometry endurance time | • FPCD: Q4 2023<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**THARROS**<br>**NCT06283966** | COPD | 5000 | • Randomised, double blind, parallel group, multi-centre event-driven trial comparing BGF MDI 320/14.4/9.6µg BID with GFF MDI 14.4/9.6µg BID in participants with COPD who are at risk of a cardiopulmonary event | • Primary endpoint: time to first severe cardiac or COPD event<br>• Secondary endpoints: time to first severe COPD exacerbation event, time to first severe cardiac event, time to cardiopulmonary death, moderate/severe COPD exacerbation rate, time to MI hospitalisation or cardiac death and time to HF acute healthcare visit/hospitalisation or cardiac death | • FPCD: Q1 2024<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# *Fasenra* (IL-5R mAb)

## Nasal polyposis and other eosinophilic diseases

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**OSTRO**<br>**NCT03401229** | Patients with severe bilateral nasal polyps who are still symptomatic despite SoC therapy; age 18 to 75 years | 413 | • Arm 1: *Fasenra* 30mg Q8W s.c.<br>• Arm 2: placebo s.c.<br>• 56-week trial<br>• Global trial – 8 countries | • Primary endpoint: effect of Fasenra on nasal polyp burden and on patient reported nasal blockage | • FPCD: Q1 2018<br>• LPCD: Q2 2019<br>• Data readout: Q3 2020<br>• Co-primary endpoints met |
| **Phase III**<br>**MANDARA**<br>**NCT04157348** | Patients with r/r EGPA on corticosteroid therapy with or without stable immunosuppressive therapy; age 18 years and older | 140 | • Arm 1: *Fasenra* 30mg Q4W s.c.<br>• Arm 2: mepolizumab 300mg Q4W s.c.<br>• 52-week trial with a minimum 1-year open label extension<br>• Global trial – 9 countries | • Primary endpoint: proportion of patients achieving remission (BVAS=0 and OCS dose ≤4mg/day) at Week 36 and Week 48 | • FPCD: Q4 2019<br>• LPCD: Q3 2022<br>• Data readout: Q3 2023<br>• Primary endpoint met |
| **Phase III**<br>**NATRON**<br>**NCT04191304** | Patients with HES (history of persistent eosinophilia >1500 cells/µL with evidence of end organ manifestations attributable to eosinophilia) and signs or symptoms of HES worsening/flare at Visit 1; age 12 years and older | 134 | • Arm 1: *Fasenra* 30mg Q4W s.c.<br>• Arm 2: placebo Q4W s.c.<br>• 24-week trial with a minimum 1-year open label extension<br>• Global trial – 15 to 18 countries | • Primary endpoint: time to first HES worsening/flare | • FPCD: Q3 2020<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# *Fasenra* (IL-5R mAb)

## Severe, uncontrolled asthma and COPD

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**RESOLUTE**<br>**NCT04053634** | Patients with moderate to very severe COPD with a history of frequent exacerbations on a background triple therapy (ICS/LABA/LAMA); age 40 to 85 years | 689 | • Double-blind, placebo-controlled trial<br>• Arm 1: *Fasenra* 100mg Q8W s.c.<br>• Arm 2: placebo Q8W s.c.<br>• 56-week treatment<br>• Global trial – 30 countries | • Primary endpoint: annualised rate of moderate or severe exacerbations over 56 weeks | • FPCD: Q4 2019<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# *Saphnelo* (type I interferon receptor mAb)

## Lupus (SLE/LN)

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**TULIP-SC**<br>**NCT04877691**<br>**Partnered (BMS)** | Moderate to severe SLE | 360 | • Arm 1: *Saphnelo* s.c.<br>• Arm 2: placebo s.c.<br>• Global trial | • Primary endpoint: BICLA at Week 52 | • FPCD: Q3 2021<br>• LPCD: Q3 2024<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**AZALEA-SLE**<br>**NCT04931563**<br>**Partnered (BMS)** | Moderate to severe SLE | 276 | • Arm 1: 300mg *Saphnelo* i.v. Q4W<br>• Arm 2: placebo i.v. Q4W<br>• Asia only | • Primary endpoint: BICLA at Week 52 | • FPCD: Q4 2021<br>• LPCD: Q2 2024<br>• Data anticipated: H1 2025 |
| **Phase III**<br>**IRIS**<br>**NCT05138133**<br>**Partnered (BMS)** | Active, proliferative LN | 360 | • Arm 1: *Saphnelo* i.v.<br>• Arm 2: placebo i.v.<br>• Global trial | • Primary endpoint: CRR at Week 52 | • FPCD: Q2 2022<br>• Data anticipated: 2026 |
| **Phase III**<br>**LAVENDER**<br>**NCT06015737**<br>**Partnered (BMS)** | Chronic and/or subacute CLE | 460 | • Arm 1: *Saphnelo* s.c.<br>• Arm 2: placebo s.c.<br>• Global trial | • Primary endpoint (US): CLA-IGA-R erythema 0/1 at Week 24<br>• Primary endpoint (EU and RoW): CLASI-70 at Week 24 | • FPCD: Q4 2024<br>• Data anticipated: >2026 |



As of 6 February 2025.
Appendix: Glossary.

# *Saphnelo* (type I interferon receptor mAb)

## Sclerosis and other myopathies

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DAISY**<br>**NCT05925803**<br>**Partnered (BMS)** | Systemic sclerosis | 306 | • Arm 1: *Saphnelo* s.c.<br>• Arm 2: placebo s.c.<br>• Global trial | • Primary endpoint: CRISS-25 at Week 52 | • FPCD: Q4 2023<br>• Data anticipated: 2026 |
| **Phase III**<br>**JASMINE**<br>**NCT06455449**<br>**Partnered (BMS)** | Idiopathic inflammatory myopathies | 240 | • Arm 1: *Saphnelo* s.c.<br>• Arm 2: placebo s.c.<br>• Global trial | • Primary endpoint: Total Improvement Score ≥40 at Week 52 | • FPCD: Q4 2024<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# *Tezspire* (TSLP mAb)

## CRSwNP, COPD and EoE

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**WAYPOINT**<br>**NCT04851964**<br>**Partnered (AMGEN)** | Severe chronic rhinosinusitis with nasal polyps; age 18 years and older | 416 | • Arm 1: *Tezspire* s.c.<br>• Arm 2: placebo s.c.<br>• 52-week trial<br>• Global trial – 10 countries | • Co-primary endpoint: nasal polyp score and participant reported nasal congestion | • FPCD: Q2 2021<br>• LPCD: Q4 2023<br>• Data readout: Q4 2024<br>• Co-primary endpoints met |
| **Phase III**<br>**CROSSING**<br>**NCT05583227**<br>**Partnered (AMGEN)** | Adult and paediatric aged 12 years and older with eosinophilic esophagitis | 360 | • Arm 1: *Tezspire* s.c. low dose<br>• Arm 2: *Tezspire* s.c. high dose<br>• Arm 3: placebo<br>• 52-week trial<br>• Global trial – 20+ countries | • Co-primary endpoints: histologic response of peak esophageal eosinophil per HPF count of ≤6 across all available esophageal levels and change from baseline in Dysphagia Symptom Questionnaire score | • FPCD: Q1 2023<br>• Data anticipated: 2026 |
| **Phase IIa**<br>**COURSE**<br>**NCT04039113**<br>**Partnered (AMGEN)** | Moderate to very severe COPD; age 40 to 80 | 338 | • Arm 1: *Tezspire* s.c.<br>• Arm 2: placebo s.c.<br>• 52-week trial<br>• Global trial – 10 countries | • Primary endpoint: rate of moderate or severe COPD exacerbations | • FPCD: Q3 2019<br>• LPCD: Q4 2022<br>• Data readout: Q2 2024<br>• Primary endpoint not met |





As of 6 February 2025.
Appendix: Glossary.

# *Tezspire* (TSLP mAb)

## Severe, uncontrolled asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**NAVIGATOR**<br>**NCT03347279**<br>**Partnered (AMGEN)** | Severe asthma; age 12 to 80 years | 1061 | • Arm 1: *Tezspire* s.c.<br>• Arm 2: placebo s.c.<br>• 52-week trial<br>• Global trial – 18 countries | • Primary endpoint: annual asthma exacerbation rate<br>• Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6) | • FPCD: Q1 2018<br>• LPCD: Q3 2019<br>• Data readout: Q4 2020<br>• Primary endpoint met |
| **Phase III**<br>**DIRECTION**<br>**NCT03927157**<br>**Partnered (AMGEN)** | Severe asthma; age 18 to 80 years | 405 | • Arm 1: *Tezspire* s.c.<br>• Arm 2: placebo s.c.<br>• 52-week trial<br>• Regional trial (Asia) – 3 countries | • Primary endpoint: annual asthma exacerbation rate<br>• Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6) | • FPCD: Q3 2019<br>• LPCD: Q2 2023<br>• Data readout: Q3 2024<br>• Primary endpoint met |





As of 6 February 2025.
Appendix: Glossary.

# HFO1234ze (next-generation propellant)

## pMDI

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III** **NCT05755932** | Mucociliary clearance in healthy volunteers | 30 | • Randomised, double-blind, multi-site, two-way crossover trial with propellant only<br>• Arm 1: HFO pMDI; 6 inhalations BID for 7 days<br>• Arm 2: HFA pMDI; 6 inhalations BID for 7 days | • Primary endpoint: change from baseline in MCC through 60 minutes following inhalation of 99m technetium sulfur colloid and gamma camera imaging<br>• Secondary endpoint: change from baseline in MCC at 3 hours following inhalation of 99m technetium sulfur colloid and gamma camera imaging | • FPCD: Q2 2023<br>• Data readout: Q4 2024 |
| **Phase III** **NCT05850494** | Well-controlled or partially-controlled asthma | 52 | • Randomised, mulit-centre double-blind, single-dose crossover trial<br>• Arm 1: HFO propellant only pMDI; 4 inhalations per dose<br>• Arm 2: HFA propellant only pMDI; 4 inhalations per dose | • Primary endpoints: change from baseline FEV1 0 to 15 minutes post-dose, cumulative incidence of bronchospasm events and safety and tolerability | • FPCD: Q2 2023<br>• Data readout: Q1 2024<br>• Primary endpoint met |
| **Phase III** **NCT06075095** | COPD | 255 | • Randomised, placebo-controlled, double-blind, multi-centre, 4-week, 3-way crossover pharmacodynamic trial to assess the equivalence of *Breztri* delivered by pMDI HFO vs. with *Breztri* delivered by MDI HFA<br>• Arm 1: *Breztri* pMDI HFO 320/14.4/9.6µg<br>• Arm 2: *Breztri* pMDI HFA 320/14.4/9.6µg<br>• Placebo: MDI HFA | • Primary endpoints: changes in FEV1 AUC (0-4) and change in morning pre-dose trough FEV1<br>• Secondary endpoints: safety and efficacy | • FPCD: Q1 2024<br>• Data anticipated: H2 2025 |
| **Phase III** **NCT06502366** | Asthma | 398 | • Randomised, placebo-controlled, double-blind, multi-centre, 12-week, 3-way, partial-replicate crossover trial<br>• BDA MDI HFO 160/180µg<br>• BDA MDI HFA 160/180µg<br>• Placebo: MDI HFA | • Primary endpoint: change from baseline in peak FEV1 in 0-60 minutes after dosing at Day 29<br>• Secondary endpoint: change from baseline in morning pre-dose trough FEV1 | • FPCD: Q3 2024<br>• Data anticipated: 2026 |
| **Phase III** **NCT05573464** | Moderate to very severe COPD | 542 | • Randomised, double-blind, 12-week (with an extension to 52 weeks in a subset of participants), parallel-group, multi-centre trial<br>• Arm 1: *Breztri* MDI HFO 160/7.2/4.8µg (2 inhalations BID)<br>• Arm 2: *Breztri* MDI HFA 160/7.2/4.8µg (2 inhalations BID) | • Primary endpoints: number of participants with AEs/SAEs and potentially clinically significant changes in Digital 12-lead Holter ECG, laboratory values, blood pressure, pulse rate, respiratory rate and body temperature | • FPCD: Q3 2022<br>• Data readout: Q4 2024 |





As of 6 February 2025.
Appendix: Glossary.

# HFO1234ze (next-generation propellant)

## pMDI

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT05477108** | Healthy volunteers | 108 | • Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial<br>• Arm 1: *Breztri* pMDI HFO 160/7.2/4.8µg (single dose of 4 inhalations)<br>• Arm 2: *Breztri* pMDI HFA 160/7.2/4.8µg (single dose of 4 inhalations) | • Primary endpoints: AUCinf, AUClast and Cmax | • FPCD: Q3 2022<br>• LPCD: Q1 2023<br>• Data readout: Q4 2023<br>• Primary endpoint met |
| **Phase I**<br>**NCT05569421** | Healthy volunteers | 108 | • Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way crossover trial<br>• Arm 1: *Breztri* pMDI HFO 160/7.2/4.8µg (single dose of 4 inhalations)<br>• Arm 2: *Breztri* pMDI HFA 160/7.2/4.8µg (single dose of 4 inhalations) | • Primary endpoints: AUCinf, AUClast and Cmax | • FPCD: Q4 2022<br>• LPCD: Q2 2023<br>• Data readout: Q1 2024<br>• Primary endpoint met |
| **Phase I**<br>**NCT06139991** | Healthy volunteers | 66 | • Randomised, double-blind, single-dose, crossover trial to assess the equivalence of *Airsupra* delivered by pMDI HFO vs. with *Airsupra* delivered by pMDI HFA<br>• Arm 1: *Airsupra* pMDI HFO 80/90µg (single dose of 2 inhalations)<br>• Arm B: *Airsupra* pMDI HFA 80/90µg (single dose of 2 inhalations) | • Primary endpoints: AUClast and Cmax | • FPCD: Q4 2023<br>• LPCD: Q2 2024<br>• Data readout: Q4 2024 |
| **Phase I**<br>**NCT06297668** | Healthy volunteers | 42 | • Randomised, partial double-blind, single dose, three-way crossover trial<br>• Arm 1: BGF MDI HFA 160/7.2/4.8µg with spacer<br>• Arm 2: BGF MDI HFO 160/7.2/4.8µg with spacer<br>• Arm 3: BGF MDI HFO 160/7.2/4.8µg without spacer | • Primary endpoints: AUClast of BGF MDI and Cmax of BGF MDI | • FPCD: Q2 2024<br>• LPCD: Q2 2024<br>• Data readout: Q4 2024 |
| **Phase I**<br>**NCT06723756** | Healthy volunteers | 105 | • Arm 1: Breztri pMDI HFO 160/14.4/4.8µg (single dose of 2 inhalations)<br>• Arm 2: Breztri pMDI HFA 160/14.4/4.8µg (single dose of 2 inhalations) | • Primary endpoints: AUClast and Cmax | • Initiating<br>• Data readout: Q3 2025 |





As of 6 February 2025.
Appendix: Glossary.

# tozorakimab (IL-33 ligand mAb)

## COPD

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**OBERON**<br>**NCT05166889** | Adults with symptomatic COPD with a history of exacerbations | 1099 | • Randomised, double-blind, placebo-controlled, parallel-group<br>• Treatment: 52-week<br>• Arm 1: tozorakimab dose 1 s.c. + SoC<br>• Arm 2: tozorakimab dose 2 s.c. + SoC<br>• Arm 3: placebo s.c. + SoC<br>• Global trial – 20 countries | • Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers)<br>• Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ | • FPCD: Q1 2022<br>• Data anticipated: 2026 |
| **Phase III**<br>**TITANIA**<br>**NCT05158387** | Adults with symptomatic COPD with a history of exacerbations | 1156 | • Randomised, double-blind, placebo-controlled, parallel-group<br>• Treatment: 52-week<br>• Arm 1: tozorakimab dose 1 s.c. + SoC<br>• Arm 2: tozorakimab dose 2 s.c. + SoC<br>• Arm 3: placebo s.c.+ SoC<br>• Global trial – 19 countries | • Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers)<br>• Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ | • FPCD: Q1 2022<br>• Data anticipated: 2026 |
| **Phase III**<br>**PROSPERO**<br>**NCT05742802** | Subjects who completed either OBERON or TITANIA will be offered the opportunity to consent (adults with symptomatic COPD with a history of exacerbations) | 1596 | • Randomised, double-blind, placebo-controlled, parallel-group, long-term extension trial<br>• Treatment: 52-weeks<br>• Arm 1: tozorakimab dose 1 s.c. + SoC<br>• Arm 2: tozorakimab dose 2 s.c. + SoC<br>• Arm 3: placebo s.c. + SoC<br>• Global trial – 38 countries | • Primary endpoint: annualised rate of severe COPD exacerbation in primary population of former smokers over the treatment period incorporating both the predecessor studies and PROSPERO<br>• Secondary endpoint: annualised rate of severe COPD exacerbation in the overall population of current and former smokers | • FPCD: Q1 2023<br>• Data anticipated: 2026 |
| **Phase III**<br>**MIRANDA**<br>**NCT06040086** | Adults with symptomatic COPD with a history of exacerbations | 1240 | • Randomised, double-blind, placebo-controlled, parallel group<br>• Arm 1: tozorakimab dose s.c. + SoC<br>• Arm 2: placebo s.c. + SoC<br>• Global trial – 29 countries | • Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers)<br>• Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers), annualised rate of severe COPD exacerbations (former and former or current smokers) and change in pre-BD FEV1, E-RS:COPD and SGRQ | • FPCD: Q4 2023<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# tozorakimab (IL-33 ligand mAb)

## Severe viral LRTD, asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**TILIA**<br>**NCT05624450** | Adults hospitalised for viral lung infection requiring supplemental oxygen | 2870 | • Randomised, double-blind, placebo-controlled, parallel group<br>• Arm 1: tozorakimab dose i.v. + SoC<br>• Arm 2: placebo i.v. + SoC<br>• Global trial – 38 countries | • Primary endpoint: progression to death or to invasive mechanical ventilation/extracorporeal membrane oxygenation<br>• Secondary endpoints: safety and other efficacy measures | • FPCD: Q4 2022<br>• Data anticipated: 2026 |
| **Phase II**<br>**FRONTIER-3**<br>**NCT04570657** | Adults with uncontrolled moderate to severe asthma | 250 | • Randomised, double-blind, placebo-controlled trial<br>• Arm 1: tozorakimab dose 1 s.c.<br>• Arm 2: tozorakimab dose 2 s.c.<br>• Arm 3: placebo s.c.<br>• Global trial – US, Argentina, Germany, Hungary, Poland, South Africa and UK | • Primary endpoint: change from baseline at Week 16 in FEV1<br>• Secondary endpoints: safety and other efficacy measures | • FPCD: Q4 2020<br>• LPCD: Q3 2022<br>• Data readout: Q2 2023<br>• Primary endpoint not met |





As of 6 February 2025.
Appendix: Glossary.

# *Beyfortus* (nirsevimab, RSV mAb-YTE)

## Infection

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**CHIMES**<br>**NCT05110261** | Healthy infants (born 29 weeks 0 days or greater gestational age) | 800 | • Randomised, double-blind, placebo-controlled<br>• Arm 1: *Beyfortus* i.m.<br>• Arm 2: placebo i.m.<br>• China only | • Primary endpoint: efficacy<br>• Secondary endpoints: safety, PK parameters and ADA | • FPCD: Q4 2021<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# *Evusheld* (AZD7442, tixagevimab + cilgavimab)

## COVID-19

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**ENDURE**<br>**NCT05375760** | Adults and pediatric individuals (≥12 years of age weighing at least 40kg) who are moderate to severely immunocompromised due to an underlying disease or are taking immunosuppressive medications and therefore unable to mount an adequate immune response | 251 | • Randomised, open-label, dose-ranging to assess safety, immunogenicity, PK and PD profiles in pre-exposure prophylaxis<br>• Arm 1: *Evusheld*, dose regimen 1<br>• Arm 2: *Evusheld*, dose regimen 2<br>• US only | • Primary endpoints: safety and tolerability, incidence of ADA<br>• Secondary endpoints: individual serum concentration; GMTs and GMFR in severe acute respiratory CoV-2 neutralizing antibodies | • FPCD: Q2 2022<br>• LPCD: Q3 2022<br>• Data readout: Q1 2024<br>• Primary endpoint met |
| **Phase I**<br>**TRUST**<br>**NCT05281601** | Pediatric participants ≥29 weeks gestational age to <18 years at increased risk of developing severe SARS-CoV-2 infection | 100 | • Open-label, single-dose, three cohort trial<br>• Cohort 1: pre-exposure prophylaxis<br>• Cohort 2: mild-to-moderate COVID-19<br>• Cohort 3: severe COVID-19<br>• *Evusheld*<br>• US only | • Primary endpoints: safety, tolerability and PK parameters | • FPCD: Q1 2022<br>• LPCD: Q1 2023<br>• Data readout: Q3 2024<br>• Primary endpoint met |





As of 6 February 2025.
Appendix: Glossary.

# *Kavigale* (sipavibart, SARS-CoV-2 LAAB)

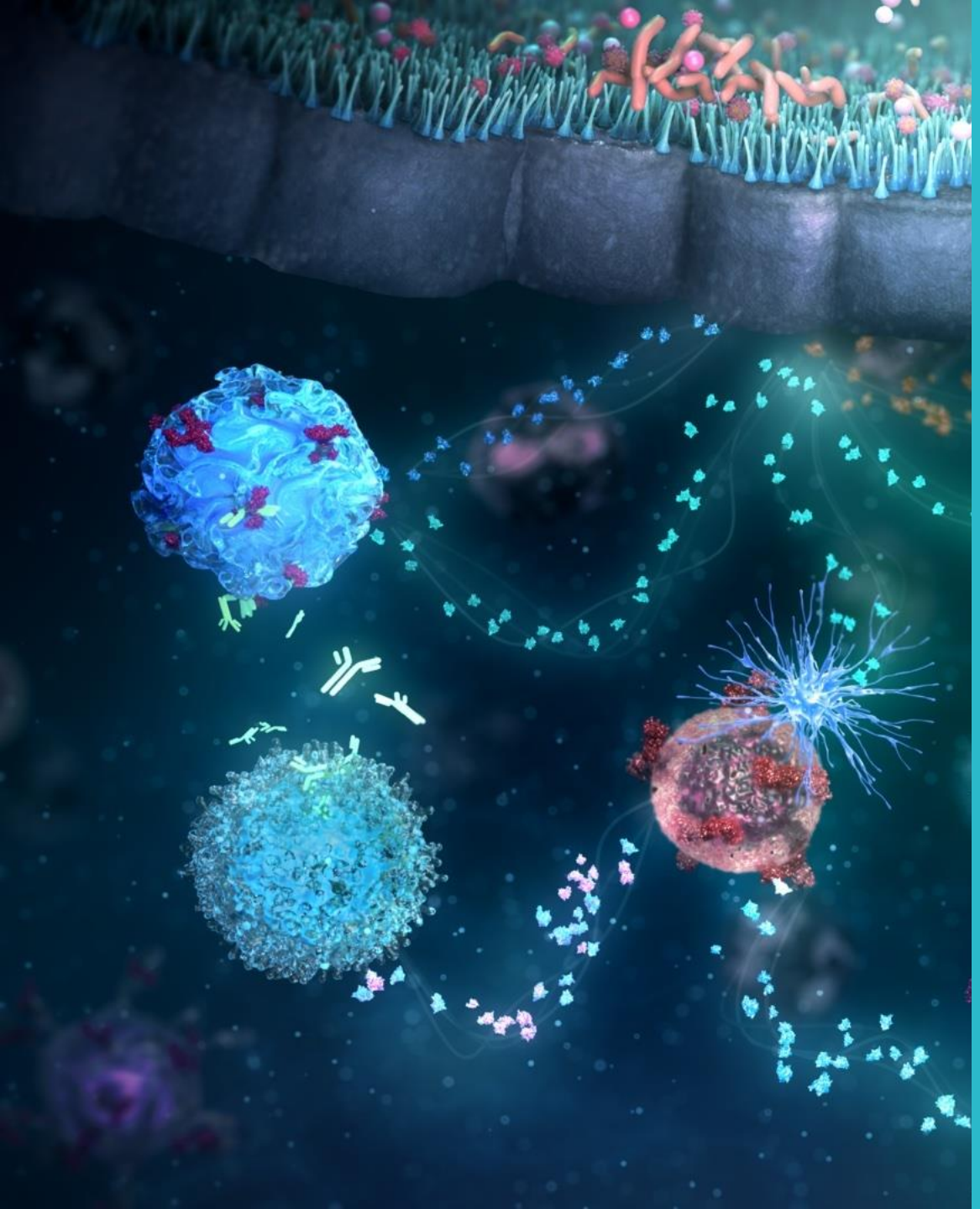
## COVID-19

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**SUPERNOVA**<br>**NCT05648110** | Phase I: healthy adults; age 18 to 55 years<br><br>Phase II: immuocompetent or immunoimpared adults<br><br>Phase III: 12 years of age or older with conditions causing immune impairment | 3200 | • 2 parts (Phase I: sentinel safety cohort and Phase III: main cohort)<br>• Phase I (sentinel safety cohort): 56 healthy adults, age 18 to 55 years, randomised in a 5:2 ratio to receive AZD5156 or placebo<br>• Phase III (main cohort): randomised 1:1 to receive AZD3152 300mg or comparator (600mg *Evusheld* or placebo) administered i.m. in the anterolateral thigh on Day 1; participants will receive a second dose of their original randomised trial intervention 6 months after Visit 1<br>• Phase II (sub-study, open-label): participants randomised 2:1 to receive 1200mg i.v. AZD3152 or 300mg i.m. *Evusheld*<br>• Global trial | • Primary endpoints (Phase III main cohort): to evaluate the safety of AZD3152 and Evusheld and/or placebo and to compare the efficacy of AZD3152 to Evusheld and/or placebo in the prevention of symptomatic COVID-19<br>• Primary endpoints (Phase II sub-study): to evaluate the safety of AZD3152 and Evushled; to compare the nAb responses to the SARS-CoV-2 to a current variant of concern following AZD3152 administration vs. SARS-CoV-2 nAb responses to prior variants following Evusheld admininistration, to characterise the PK of AZD3152 and Evusheld in serum and to evaluate the ADA responses to AZD3152 and AZD7442 in serum | • FPCD: Q4 2022<br>• LPCD: Q4 2023<br>• Data readout: Q2 2024<br>• Primary Endpoint met |
| **Phase I**<br>**LITTLE DIPPER**<br>**NCT05872958** | Healthy adult participants; age 18 to 55 years | 96 | • Phase I, double-blind, placebo-controlled, multi-centre, dose exploration trial<br>• to evaluate the safety and PK of AZD3152 in healthy adult participants across different dose levels and routes of administration<br>• participants randomised in a 10:2 ratio to receive either AZD3152 or placebo administered i.m. or i.v. across 5 fixed-dose cohorts | • Primary endpoint: to evaluate the safety of i.m. or i.v. administration of AZD315 and to characterise the PK of AZD3152 in serum after a single i.m. or i.v. dose<br>• Secondary endpoint: to evaluate ADA responses to AZD3152 | • FPCD: Q2 2023<br>• LPCD: Q3 2023<br>• Data readout: Q4 2023<br>• Primary endpoint met |





As of 6 February 2025.
Appendix: Glossary.



# BioPharmaceuticals: early-stage development

AstraZeneca

# AZD0233 (oral CX3CR1)

## Dilated cardiomyopathy

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06381466** | Healthy volunteers | 96 | • Randomised, SAD/MAD dose escalating trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters | • FPCD: Q2 2024<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD0780 (PCSK9 inhibitor)

## Dyslipidaemia

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**PURSUIT**<br>**NCT06173570** | Dyslipidaemia | 428 | • Randomised trial with equal distribution across five parallel treatment arms to either placebo or one of four AZD0780 doses | • Primary endpoint: percent change in LDL-C level from baseline to Week 12<br>• Secondary endpoints: percent change from baseline of LDL-C at Week 12, plasma concentrations summarised by sampling timepoint, percent change from baseline at Week 12 in other lipid parameters and inflammatory markers and safety and tolerability | • FPCD: Q1 2024<br>• LPCD: Q2 2024<br>• Data anticipated: H1 2025 |
| **Phase II**<br>**NCT06692764** | Participants with ASCVD or risk equivalents and LDL-C ≥70 mg/dL on stable medication | 172 | • Phase II, multi-centre, randomised, double-blind, placebo-controlled, crossover trial | • Primary endpoint: ambulatory 24-hour average systolic blood pressure at Week 4<br>• Secondary endpoint: ambulatory 24-hour average diastolic blood pressure at Week 4 | • FPCD: Q4 2024<br>• Data anticipated: H1 2025 |



As of 6 February 2025.
Appendix: Glossary.

# AZD0780 (PCSK9 inhibitor)

## Dyslipidaemia

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I** NCT05384262 | Healthy volunteers | 183 | • Randomised, placebo-controlled SAD/MAD trial | • Primary endpoints: safety and tolerability | • FPCD: Q2 2022<br>• LPCD: Q2 2024<br>• Data readout: Q4 2024 |
| **Phase I** NCT05787002 | Healthy volunteers | 16 | • Open-label, two-period, two-sequence crossover trial to assess the effect of AZD0780 on the PK of *Crestor* | • Primary endpoints: PK parameters, safety and tolerability | • FPCD: Q1 2023<br>• LPCD: Q2 2023<br>• Data readout: Q4 2023 |
| **Phase I** NCT05817461 | Healthy volunteers | 8 | • Open-label, two-part sequential human ADME trial | • Primary endpoints: mass balance recovery, absorption, metabolism, excretion of [14C]AZD0780 and absolute bioavailability of AZD0780<br>• Secondary endpoints: safety and tolerability | • FPCD: Q2 2023<br>• LPCD: Q2 2023<br>• Data readout: Q4 2023 |
| **Phase I** NCT06576765 | Hepatic impairment and matched healthy controls | 32 | • Multi-centre, single-dose, non-randomised, open-label, parallel-group trial | • Primary endpoint: PK parameters<br>• Secondary endpoints: safety and tolerability | • FPCD: Q3 2024<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I** NCT06592482 | Renal impairment and matched healthy controls | 42 | • Multi-centre, single-dose, non-randomised, open-label, parallel-group trial | • Primary endpoint: PK parameters<br>• Secondary endpoints: safety and tolerability | • FPCD: Q3 2024<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I** NCT06671405 | Healthy volunteers | 78 | • Open-label, fixed sequence trial to assess the PK of AZD0780 when administered in combination with itraconazole, carbamazepine, and the PK of midazolam and EE/LNG when administered with AZD0780 | • Primary endpoint: PK parameters<br>• Secondary endpoints: safety and PK parameters | • FPCD: Q4 2024<br>• Data anticipated: H2 2025 |
| **Phase I** NCT06742853 | Healthy volunteers with elevated LDL-C | 120 | • Randomised, single-blind, placebo-controlled | • Primary endpoints: percent change in LDL-C at Week-4 and safety and tolerability | • FPCD: Q4 2024<br>• Data anticipated: H2 2025 |



As of 6 February 2025.
Appendix: Glossary.

# AZD1705 (Angptl3 inhibitor)

## Dyslipidaemia

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06238466** | Dyslipidaemia | 112 | • Part A: single dose of AZD1705 with an in-clinic period of 3 days followed by an outpatient follow-up period of approximately 16 weeks<br>• Part B: 2 doses of AZD1705 given 28 days apart with an in-clinic period followed by an outpatient follow-up period of approximately 20 weeks | • Primary endpoints: AEs and SAEs<br>• Secondary endpoints: AUCinf, AUClast, Cmax, Ae, fe, CLR, LDL-C, ApoB, triglycerides and target plasma protein | • FPCD: Q1 2024<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD2373 (APOL1)

## Chronic kidney disease

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT04269031** | Healthy volunteers | 30 | • SAD dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort<br>• Arm 1: AZD2373 s.c.<br>• Arm 2: placebo s.c.<br>• US only | • Primary endpoints: safety and tolerability<br>• Secondary endpoint: PK parameters | • FPCD: Q1 2020<br>• LPCD: Q3 2021<br>• Data readout: Q3 2022 |
| **Phase I**<br>**NCT05351047** | Healthy volunteers | 24 | • MAD dose escalation in 3 cohorts with 6 volunteers per cohort receiving AZD2373 and 2 volunteers per cohort receiving placebo<br>• Arm 1: AZD2373 s.c.<br>• Arm 2: placebo s.c.<br>• US only | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters, effect of s.c. MAD administrations of AZD2373 on plasma concentrations of APOL1 protein and APOL1 G0, G1, G2 allele genotype status in trial participants | • FPCD: Q2 2022<br>• LPCD: Q1 2023<br>• Data readout: Q4 2023 |





As of 6 February 2025.
Appendix: Glossary.

# AZD2389 (anti-fibrotic mechanism)

## MASH

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**BORANA**<br>**NCT06750276** | Participants with liver fibrosis and compensated cirrhosis | 36 | • Randomised, single-blind, placebo-controlled trial | • Primary endpoints: safety and tolerability | • FPCD: Q4 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT06138795** | Healthy volunteers | 104 | • Randomised, placebo-controlled SAD/MAD trial | • Primary endpoints: safety and tolerability | • FPCD: Q4 2023<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD2693 (PNPLA3 ASO)

## MASH

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIb**<br>**FORTUNA**<br>**NCT05809934** | NASH with fibrosis | 180 | • Randomised, double-blind, placebo-controlled, multi-centre trial<br>• Arm 1: AZD2693 s.c. dose 1<br>• Arm 2: AZD2693 s.c. dose 2<br>• Arm 3: placebo s.c.<br>• Global trial | • Primary endpoints: efficacy, safety and tolerability | • FPCD: Q2 2023<br>• LPCD: Q3 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT04483947** | NASH/NAFLD F0-F3 | 74 | • MAD with 4 cohorts receiving AZD2693 and placebo in each cohort<br>• Arm 1: AZD2693 s.c.<br>• Arm 2: placebo s.c.<br>• US only | • Primary endpoints: safety and tolerability<br>• Secondary endpoint: PK parameters | • FPCD: Q2 2021<br>• LPCD: Q3 2023<br>• Data readout: Q2 2024 |
| **Phase I**<br>**NCT05107336** | Healthy volunteers | 44 | • MAD with 4 cohorts receiving AZD2693 and placebo in each cohort<br>• Arm 1: AZD2693 s.c.<br>• Arm 2: placebo s.c.<br>• JP only | • Primary endpoints: safety and tolerability<br>• Secondary endpoint: PK parameters | • FPCD: Q4 2021<br>• LPCD: Q4 2022<br>• Data readout: Q4 2023 |
| **Phase I**<br>**NCT05919069** | Hepatic impairment | 32 | • Single-dose, non-randomised, open-label, parallel group trial<br>• US only | • Primary endpoints: safety, tolerability and PK parameters | • FPCD: Q3 2023<br>• LPCD: Q2 2024<br>• Data readout: Q4 2024 |





As of 6 February 2025.
Appendix: Glossary.

# AZD3427 (relaxin)

## Heart failure

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**Re-PHiRE**<br>**NCT05737940** | HF and pulmonary hypertension due to left heart disease | 220 | • Randomised, double-blind, placebo-controlled, multi-centre trial<br>• Arm 1: AZD3427 (high dose)<br>• Arm 2: AZD3427 (medium dose)<br>• Arm 3: AZD3427 (low dose)<br>• Arm 4: placebo<br>• Global trial – US, Canada, China, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Austria, Germany, Denmark and Sweden | • Primary endpoint: change in PVR from baseline to Week 25 vs. placebo as measured by right heart catheterisation | • FPCD: Q2 2023<br>• Data anticipated: H2 2025 |
| **Phase Ib**<br>**RE-PERFUSE**<br>**NCT06611423** | HFrEF patients with mild renal impairment | 12 | • Eligible participants randomised equally<br>• Arm 1: i.v. saline placebo followed by s.c. AZD3427<br>• Arm 2: i.v. saline placebo followed by s.c. AZD3427 placebo<br>• Arm 3: i.v. dopamine diluted in saline followed by s.c. AZD3427<br>• Arm 4: i.v. dopamine diluted in saline followed by s.c. AZD3427 placebo | • Primary endpoint: volumetric fraction of the renal cortex with increased perfusion from baseline to Day 8 compared to placebo as measured using PET | • FPCD: Q4 2024<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD4144 (inflammation modulator)

## Cardiorenal disease

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06122714** | Healthy volunteers | 95 | • Randomised, single-blind, placebo-controlled, SAD/MAD sequential group trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters | • FPCD: Q4 2023<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT06491550** | Healthy volunteers | 92 | • Randomised, single-blind, placebo-controlled, SAD/MAD sequential group trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters | • FPCD: Q3 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT06693765** | Participants with renal impairment, end-stage kidney disease and healthy volunteers | 24 | • Single-dose, non-randomised, open-label, parallel-group trial | • Primary endpoints: safety, tolerability and PK parameters | • FPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT06675175** | Participants with established ASCVD | 28 | • Randomised, double-blind, placebo-controlled, parallel group trial | • Primary endpoints: safety, tolerability and PD parameters<br>• Secondary endpoints: PK and PD parameters | • FPCD: Q1 2025<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD5004 (oral GLP-1 RA)

## Type 2 diabetes, obesity

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIb<br>SOLSTICE<br>NCT06579105** | Type 2 diabetes | 384 | • Arm 1: AZD5004 tablet<br>• Arm 2: AZD5004 tablet<br>• Arm 3: AZD5004 tablet<br>• Arm 4: AZD5004 tablet<br>• Arm 5: AZD5004 tablet<br>• Arm 6: AZD5004 tablet<br>• Arm 7: active comparator semaglutide tablet<br>• Arm 8: placebo matching AZD5004 tablet<br>• Global trial | • Primary endpoint: change in HbA1c from baseline at 26 weeks<br>• Secondary endpoints: change in fasting glucose from baseline, proportion of participants achieving HbA1c ≤6.5% and <7.0% and percent change in body weight from baseline | • FPCD: Q4 2024<br>• Data anticipated: 2026 |
| **Phase IIb<br>VISTA<br>NCT06579092** | Obesity or overweight who have at least one weight-related comorbidity | 304 | • Arm 1: AZD5004 tablet<br>• Arm 2: AZD5004 tablet<br>• Arm 3: AZD5004 tablet<br>• Arm 4: AZD5004 tablet<br>• Arm 5: AZD5004 tablet<br>• Arm 6: placebo matching AZD5004 tablet<br>• Global trial | • Primary endpoints: percent change in body weight from baseline at 26 weeks and proportion of participants with weight loss ≥5% from baseline weight at 26 weeks<br>• Secondary endpoints: percent change in body weight from baseline at 36 weeks, proportion of participants with weight loss ≥5% and absolute change from baseline in body weight at 36 weeks | • FPCD: Q4 2024<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# AZD5004 (oral GLP-1 RA)

## Type 2 diabetes, obesity

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06555822** | Healthy volunteers | 31 | • Part A – Arm 1: AZD5004 oral tablet<br>• Part A – Arm 2: placebo oral tablet<br>• Part B: single dose, open label crossover | • Primary endpoints (Part A): safety and tolerability<br>• Secondary endpoints (Part A): PK and PD parameters<br>• Primary endpoint (Part B): PK parameters<br>• Secondary endpoints (Part B): safety and tolerability | • FPCD: Q3 2024<br>• Data anticipated: H2 2025 |
| **Phase I**<br>**NCT06703658** | Healthy volunteers or participants with type 2 diabetes mellitus | 36 | • SAD: 3 cohorts to receive AZD5004 or placebo tablet<br>• MAD: 1 cohort to receive AZD5004 or placebo tablet<br>• Japan only | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK and PD parameters | • FPCD: Q4 2024<br>• Data anticipated: H2 2025 |
| **Phase I**<br>**NCT06742762** | Healthy volunteers or participants with renal impairment | 21 | • Multi-centre, single-dose, non-randomised, open-label, parallel-group trial | • Primary endpoints: PK parameters<br>• Secondary endpoints: safety and tolerability | • FPCD: Q1 2025<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD5462 (oral relaxin)

## Heart failure

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIb**<br>**LUMINARA**<br>**NCT06299826** | Stable patients with chronic heart failure | 360 | • Two cohort, randomised, double-blind, placebo-controlled, multi-centre trial<br>• Arm 1: AZD5462 (high dose)<br>• Arm 2: AZD5462 (medium dose)<br>• Arm 3: AZD5462 (low dose)<br>• Arm 4: placebo<br>• Global trial | • Primary endpoint: change in heart function from baseline to Week 25 compared to placebo | • FPCD: Q3 2024<br>• Data anticipated: H2 2025 |
| **Phase Ib**<br>**AURORA**<br>**NCT06639087** | Stable patients with heart failure and moderately impaired renal function | 40 | • Randomised, double-blind, placebo-controlled, multi-centre mechanistic trial<br>• Arm 1: AZD5462 + dapagliflozin<br>• Arm 2: placebo + dapagliflozin | • Primary endpoint: change in fractional excretion of sodium from baseline to Day 1 | • FPCD: Q4 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT04994106** | Healthy volunteers | 98 | • Single-centre SAD and MAD<br>• Part A: SAD (8 cohorts)<br>• Arm 1: AZD5462<br>• Arm 2: placebo<br>• Part B: MAD (5 cohorts)<br>• Arm 1: AZD5462<br>• Arm 2: placebo<br>• US only | • Primary endpoints: safety and tolerability | • FPCD: Q4 2021<br>• LPCD: Q3 2022<br>• Data readout: Q2 2023 |
| **Phase I**<br>**GLITTER**<br>**NCT06661733** | Moderate or severe renal impairment and healthy volunteers | 25 | • Single centre, non-randomised, open-label, parallel group trial<br>• Cohort 1: AZD5462<br>• Cohort 2: AZD5462<br>• Cohort 3: AZD5462 | • Primary endpoints: PK parameters, safety and tolerability | • FPCD: Q4 2024<br>• LPCD: Q1 2025<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD6234 (long-acting amylin)

## Obesity with related co-morbidities

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**APRICUS**<br>**NCT06595238** | Participants living with obesity or overweight with co-morbidity | 231 | • Randomised, double-blind, placebo-controlled trial | • Primary endpoints: percent change in body weight from baseline to Week 26 and weight loss ≥5% from baseline weight to Week 26 | • FPCD: Q4 2024<br>• Data anticipated: H2 2025 |
| **Phase I**<br>**NCT05511025** | Healthy participants who are overweight or obese | 64 | • SAD trial | • Primary endpoint: safety | • FPCD: Q4 2022<br>• Data readout: Q1 2024 |
| **Phase I**<br>**NCT06132841** | Overweight or obese participants | 142 | • Randomised, single-blind, placebo-controlled trial with repeated doses of AZD6234 or placebo via s.c. injection | • Primary endpoints: safety and tolerability of repeat doses | • FPCD: Q4 2023<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# AZD9550 (GLP-1-glucagon agonist)

## MASH

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT05848440** | Healthy volunteers | 64 | • SAD trial | • Primary endpoints: safety and tolerability | • FPCD: Q2 2023<br>• LPCD: Q4 2023<br>• Data readout: Q2 2024 |
| **Phase I**<br>**CONTEMPO**<br>**NCT06151964** | Overweight and obese participants with T2DM | 90 | • Randomised, single-blind, placebo-controlled, MAD trial with 4 parts (A to D)<br>• Part A: multiple repeat doses of AZD9550 or placebo given as 4 QW s.c. doses for 4 weeks to 2 sequential cohorts evaluating 2 low dose levels of AZD9550 or placebo<br>• Part B: QW up-titration over 5 doses of AZD9550 or placebo<br>• Part C: bi-weekly/monthly up-titration of AZD9550 or placebo for 24 weeks<br>• Part D: bi-weekly/monthly up-titration of AZD9550 or placebo for 24 weeks (Japan only) | • Primary endpoints: safety, tolerability and PK parameters | • FPCD: Q4 2023<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# mitiperstat (MPO inhibitor)

## Cardiovascular disease, MASH

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIb**<br>**ENDEAVOR**<br>**NCT04986202** | HFpEF | 711 | • Randomised, double-blind trial<br>• Arm 1: 2.5mg mitiperstat<br>• Arm 2: 5mg mitiperstat<br>• Arm 3: placebo<br>• Global trial | • Primary endpoints: safety and efficacy | • FPCD: Q3 2021<br>• Data readout: Q2 2024<br>• Trial discontinued due to strategic portfolio priorisation |
| **Phase II**<br>**COSMOS**<br>**NCT05638737** | NASH | 90 | • Randomised, placebo-controlled, double-blind<br>• Arm 1: 5mg mitiperstat<br>• Arm 2: placebo<br>• Global trial | • Primary endpoints: safety, tolerability and PD parameters | • FPCD: Q1 2023<br>• Data readout: Q2 2024<br>• Trial discontinued due to strategic portfolio priorisation |
| **Phase I**<br>**NCT05751759** | Participants with hepatic impairment and participants with normal hepatic function | 32 | • Phase I, single dose, non-randomised, open-label, parallel-group trial | • Primary endpoints: safety, tolerability and PK parameters | • FPCD: Q1 2023<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025<br>• Trial discontinued due to strategic portfolio priorisation |





As of 6 February 2025.
Appendix: Glossary.

# atuliflapon (FLAP inhibitor)

## Asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIa**<br>**FLASH**<br>**NCT05251259** | Patients with moderate-to-severe uncontrolled asthma | 666 | • Randomised, placebo-controlled, double-blind, multi-centre trial with a lead-in PK cohort<br>• PK cohort<br>• Arm 1: atuliflapon<br>• Arm 2: placebo<br>• Part 1<br>• Arm 1: atuliflapon<br>• Arm 2: placebo<br>• Global trial | • Primary endpoint: time to first CompEx asthma event | • FPCD: Q2 2022<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# AZD0120 (GC012F, autologous anti-CD19 and anti-BCMA CAR-T)

## Lupus (SLE)

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/II**<br>**NCT06530849** | Refractory systemic lupus erythematosus | 21 | • Single-arm, open label, multi-centre trial | • Primary endpoint (Phase I): safety at 28 days<br>• Primary endpoint (Phase II): efficacy (SRI-4 response) at Week 48 | • FPCD: Q3 2024<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# AZD1163 (bispecific antibody)

## Rheumatoid arthritis

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06103877** | Healthy volunteers | 108 | • Randomised, double-blind, placebo-controlled SAD/MAD trial<br>• Part 1 (SAD): 9 cohorts with 8 i.v. administered dose levels and 1 s.c. administered dose level of AZD1163<br>• Part 2 (MAD): 2 s.c. dose levels of AZD1163 | • Primary endpoint: number of participants with AEs<br>• Secondary endpoints: AUCinf, AUClast and Cmax | • FPCD: Q4 2023<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# AZD4604 (inhaled JAK-1 inhibitor)

## Asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIa**<br>**AJAX**<br>**NCT06020014** | Moderate-to-severe asthma uncontrolled on medium-to high-dose ICS-LABA | 320 | • Multi-centre, randomised, placebo-controlled, double-blind, parallel-group trial<br>• Arm 1: AZD4604<br>• Arm 2: placebo | • Primary endpoint: time to first CompEx asthma event<br>• Secondary endpoints: Pre-BD FEV1, CAAT, ACQ-6, average morning and average evening PEF, daily asthma symptom score, time to first CompEx acute worsening event, CompEx event rate and CompEx acute worsening event rate | • FPCD: Q4 2023<br>• Data anticipated: 2026 |
| **Phase IIa**<br>**ARTEMISIA**<br>**NCT06435273** | Adult patients with moderate-to-severe asthma receiving treatment with medium-to-high dose ICS-LABA | 48 | • Multi-centre, randomised, placebo-controlled, double-blind, parallel-group trial<br>• Arm 1: AZD4604<br>• Arm 2: placebo | • Primary endpoint: gene expression in airway epithelial cells<br>• Secondary endpoints: STAT phosphorylation and cellular pathology | • FPCD: Q3 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT04769869** | Healthy volunteers and patients with mild asthma | 137 | • SAD/MAD/POM trial<br>• Part 1 SAD<br>• Arm 1: AZD4604 (DPI)<br>• Arm 2: placebo (DPI)<br>• Part 2 MAD<br>• Arm 1: AZD4604 (DPI)<br>• Arm 2: placebo (DPI)<br>• Part 3 POM<br>• Arm 1: AZD4604 (DPI)<br>• Arm 2: placebo (DPI)<br>• UK only | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and FENO | • FPCD: Q4 2021<br>• Data readout: Q3 2023 |
| **Phase I**<br>**NCT06519968** | Healthy volunteers | | • Part 1a: SAD cohorts in healthy Japanese participants<br>• Part 1b: multiple dose cohort in healthy Japanese participants<br>• Part 2a: SAD cohort in healthy Chinese participants<br>• Part 2b: multiple dose cohort in healthy Chinese participants | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters | • FPCD: Q3 2024<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD6793 (IRAK4)

## Inflammatory diseases

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT05662033** | Healthy volunteers | 133 | • Single-blind, randomised, placebo-controlled trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoint: PK parameters | • FPCD: Q4 2022<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT06368440** | Healthy volunteers | 40 | • Single-blind, randomised, placebo-controlled trial<br>• Japanese and Chinese healthy participants | • Primary endpoint: safety<br>• Secondary endpoints: PK parameters | • FPCD: Q2 2024<br>• LPCD: Q4 2024<br>• Data anticipated: H1 2025 |
| **Phase I**<br>**NCT06494644** | Healthy participants | 17 | • A single-group trial with a duration of up to 8 weeks (maximum of 53 days) including Screening, Period 1, Period 2, Period 3 and Follow-up to assess the pharmacokinetics of AZD6793 when administered alone and in combination with itraconazole in healthy participants | • Primary endpoint: PK parameters (Cmax, AUC, CL/F, t1/2, tmax, Vz/F, RAUC)<br>• Secondary endpoint: safety | • FPCD: Q3 2023<br>• LPCD: Q4 2023<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD6912 (siRNA)

## Rheumatoid arthritis

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06115967** | Healthy volunteers | 64 | • Randomised, double-blind, placebo-controlled SAD trial<br>• Arm 1: AZD6912<br>• Arm 2: placebo | • Primary endpoint: incidence of AEs<br>• Secondary endpoint: PK parameters | • FPCD: Q4 2023<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# AZD7798 (humanised mAb)

## Crohn's disease

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIa**<br>**AMALTHEA**<br>**NCT06450197** | Moderate to severe Crohn's disease | 192 | • Randomised, double-blind, placebo-controlled trial<br>• Arm 1: AZD7798<br>• Arm 2: placebo | • Primary endpoint: Crohn's Disease Activity Index (CDAI) remission<br>• Secondary endpoints: endoscopic response, endoscopic remission, endoscopic score change from baseline, CDAI response, CDAI score change from baseline, symptomatic remission, PK parameters and ADA | • FPCD: Q4 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT05452304** | Global, Japanese and Chinese healthy volunteers | 144 | • SAD, repeating dose trial<br>• Arm 1: AZD7798<br>• Arm 2: placebo<br>• s.c. and i.v. administration<br>• UK only | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and immunogenicity | • FPCD: Q3 2022<br>• LPCD: Q3 2024<br>• Data readout: Q4 2023 |





As of 6 February 2025.
Appendix: Glossary.

# AZD8630 (inhaled TSLP)

## Asthma

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**LEVANTE**<br>**NCT06529419**<br>**Partnered (AMGEN)** | Adults with uncontrolled asthma at risk of exacerbations | 516 | • Randomised, placebo-controlled, double-blind, dose range-finding, multi-centre trial<br>• Arm 1: AZD8630 Dose A<br>• Arm 2: AZD8630 Dose B<br>• Arm 3: AZD8630 Dose C<br>• Arm 4: placebo | • Primary endpoint: time to first CompEx asthma event<br>• Secondary endpoints: change from baseline in pre-bronchodilator forced expiratory volume in 1 second and safety and tolerability | • FPCD: Q3 2024<br>• Data anticipated: 2026 |
| **Phase I**<br>**NCT05110976**<br>**Partnered (AMGEN)** | Healthy volunteers and patients with asthma | 232 | • SAD and MAD trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK parameters and FENO | • FPCD: Q1 2022<br>• LPCD: Q3 2023<br>• Data readout: Q4 2023 |
| **Phase I**<br>**NCT06531811**<br>**Partnered (AMGEN)** | Healthy volunteers | 28 | • Randomised, open-label, 2-treatment, 2-period trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoint: PK parameters | • FPCD: Q3 2024<br>• LPCD: Q3 2024<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD8965 (arginase enzyme inhibitor)

## IPF

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06502379** | Healthy participants | 163 | • Randomised, single-blind, SAD/MAD, placebo-controlled, AZD8965/placebo administered orally<br>• Part 1: SAD cohorts<br>• Part 2: MAD cohorts<br>• Part 3a: Japanese and Chinese participants SAD cohorts<br>• Part 3b: Japanese and Chinese participants SMAD cohorts<br>• Part 4: food effect cohort | • Primary endpoints (Part 1, 2, 3): safety and tolerability measures<br>• Primary endpoint (Part 4): PK parameters<br>• Secondary endpoint (Part 1, 2, 3): PK parameters<br>• Secondary endpoints (Part 4): safety and tolerability measures under fasted and fed condition | • FPCD: Q3 2024<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# mitiperstat (MPO inhibitor)

## COPD

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**CRESCENDO**<br>**NCT05492877** | Moderate to severe COPD; age 40 to 80 | 381 | • Randomised, double-blind trial<br>• Arm 1: 5mg mitiperstat<br>• Arm 2: placebo<br>• Global trial – 14 countries | • Primary endpoint: time to first COPD CompEx event<br>• Secondary endpoints: plasma concentration-time profiles, PK parameters, time to first COPD exacerbation event, post-BD FEV1, respiratory symptoms, disease impact, safety and tolerability | • FPCD: Q1 2023<br>• LPCD: Q3 2024<br>• Trial discontinued due to strategic portfolio priorisation |



As of 6 February 2025.
Appendix: Glossary.

# AZD4041 (orexin 1 receptor antagonist)

## Opioid use disorder

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**NCT06406400** | Healthy volunteers and opioid users | 100 | • Part 1: open label, fixed sequence trial of AZD4041 and itraconazole<br>• Part 2: randomised placebo-controlled double-blind trial | • Primary endpoints (Part 1): DDI, PK parameters and safety<br>• Primary endpoints (Part 2): efficacy, safety, PK and PD parameters | • FPCD: Q2 2024<br>• Trial discontinued due to safety |
| **Phase I**<br>**NCT05587998**<br>**Partnered (National Institute on Drug Abuse)** | Healthy recreational opioid users | 36 | • Randomised, double-blind, placebo-controlled, fixed sequence trial | • Primary endpoint: change in respiratory parameters | • FPCD: Q3 2022<br>• LPCD: Q2 2023<br>• Data readout: Q3 2023<br>• Primary endpoint met |





As of 6 February 2025.
Appendix: Glossary.

# MEDI0618 (PAR2 antagonist mAb)

## Migraine prevention

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**AURORA**<br>**NCT06602479** | Cohort of participants failed >3 small molecule migraine treatments and eligible to receive aCGRP therapy (aCGRP-N)<br>Cohort of particpants who have failed one or more aCGRP therapies (aCGRP-IR) | 408 | • Arm 1: CGRP-N MEDI0618 Dose A<br>• Arm 2: CGRP-N placebo comparator<br>• Arm 3: CGRP N - MEDI0618 Dose B<br>• Arm 4: CGRP N - MEDI0618 Dose C<br>• Arm 5: CGRP N - MEDI0618 Dose D<br>• Arm 6: CGRP IR - MEDI0618 Dose A<br>• Arm 7: CGRP IR - placebo comparator<br>• Global trial | • Primary endpoint: change in number of migraine headache days (MHD) from 4-week baseline to last 4 weeks of treatment period<br>• Secondary endpoints: participants with at least 50% reduction in number of MHDs in the last 4 weeks of treatment period compared to 4-week baseline, change in MIDAS score from baseline to end of treatment period and to follow-up, change in number of moderate or severe MHDs from 4-week baseline to last 4 weeks of treatment period and change in number of moderate or severe headache days from 4-week baseline to last 4 weeks of treatment period and change in frequency of use of permitted acute treatment to abort migraine headaches from 4-week baseline to last 4 weeks of the treatment period | • FPCD: Q4 2024<br>• Data anticipated: 2026 |



As of 6 February 2025.
Appendix: Glossary.

# MEDI0618 (PAR2 antagonist mAb)

## Osteoarthritis pain, migraine prevention

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT05714254** | Healthy volunteers | 112 | • Randomised, double-blind, placebo-controlled MAD trial<br>• Arm 1: MEDI0618 i.v. or placebo<br>• Arm 2: MEDI0618 s.c. or placebo | • Primary endpoints: safety, tolerability and PK parameters | • FPCD: Q4 2022<br>• LPCD: Q3 2023<br>• Data readout: Q1 2024 |





As of 6 February 2025.
Appendix: Glossary.

# MEDI7352 (NGF TNF bispecific mAb)

## Osteoarthritis pain

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase IIb**<br>**NCT04675034** | Painful osteoarthritis of the knee | 350 | • MAD trial<br>• Arm 1: MEDI7352 s.c.<br>• Arm 2: placebo s.c.<br>• Global – 7 countries | • Primary endpoint: dose response<br>• Secondary endpoints: safety, tolerability, PK and PD parameters and ADA | • FPCD: Q1 2021<br>• LPCD: Q3 2022<br>• Data readout: Q4 2023 |
| **Phase IIa**<br>**NCT03755934** | Painful diabetic neuropathy | 107 | • MAD trial<br>• Arm 1: MEDI7352 i.v.<br>• Arm 2: placebo i.v.<br>• Europe only | • Primary endpoint: dose response<br>• Secondary endpoints: safety, tolerability and PK and PD parameters | • FPCD: Q4 2018<br>• LPCD: Q1 2023<br>• Data readout: Q4 2023 |





As of 6 February 2025.
Appendix: Glossary.

# AZD0292 (Psl-PcrV N3Y-bispecific mAb)

## Bronchiectasis

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06311760** | Healthy volunteers | 32 | • Randomised, single-blind, placebo-controlled trial<br>• Arm 1: AZD0292 Dose 1 administered via i.v. infusion<br>• Arm 2: AZD0292 Dose 2 administered via i.v. infusion<br>• Arm 3: AZD0292 Dose 3 administered via i.v. infusion<br>• Arm 4: AZD0292 Dose 4 administered via i.v. infusion<br>• Arm 5: placebo administered via i.v. infusion | • Primary endpoints: AEs and participants with AESI<br>• Secondary endpoints: Cmax, AUClast, AUCinfinity and ADA | • FPCD: Q2 2024<br>• LPCD: Q3 2024<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD5148 (anti-TcdB mAb)

## Clostridium difficile

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**NCT06469151** | Healthy volunteers | 84 | • Randomised, double-blind, placebo-controlled, dose escalation<br>• Cohort 1: AZD5148 (dose 1, i.m.) or placebo<br>• Cohort 2a: AZD5148 (dose 2, i.m.) or placebo<br>• Cohort 2b: AZD5148 (dose 2, i.m., Chinese participants) or placebo<br>• Cohort 3: AZD5148 (dose 2, i.v.) or placebo<br>• Cohort 4a: AZD5148 (dose 3, i.v.) or placebo<br>• Cohort 4b: AZD5148 (dose 3, i.v., Chinese participants) or placebo<br>• Cohort 5: AZD5148 (dose 4, i.v.) or placebo | • Primary endpoint: safety<br>• Secondary endpoint: PK parameters | • FPCD: Q2 2024<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# AZD7760 (mAb combination targeting S aureus virulence factors)

## Prevention of Staph aureus infection

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I/IIa**<br>NCT06749457 | Phase I: healthy volunteers male and female participants aged 18 to 55 years<br>Phase IIa: patients with ESKD receiveing heamodialysis through a central vensous catheter | 231 | • Phase I: randomised, double-blind, placebo-controlled, dose escalation study to evaluate the safety and PK of AZD7760 to evaluate 3 doses<br>• Phase IIa: randomised, double-blind, placebo-controlled trial to evaluate the safety and PK of AZD7760 | • Primary endpoint (Phase I): safety<br>• Primary endpoint (Phase IIa): safety<br>• Secondary endpoints (Phase I): PK parameters and ADA<br>• Secondary endpoints (Phase Iia): PA parameters, ADA and D451 safety | • FPCD: Q1 2025<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# mRNA VLP vaccine

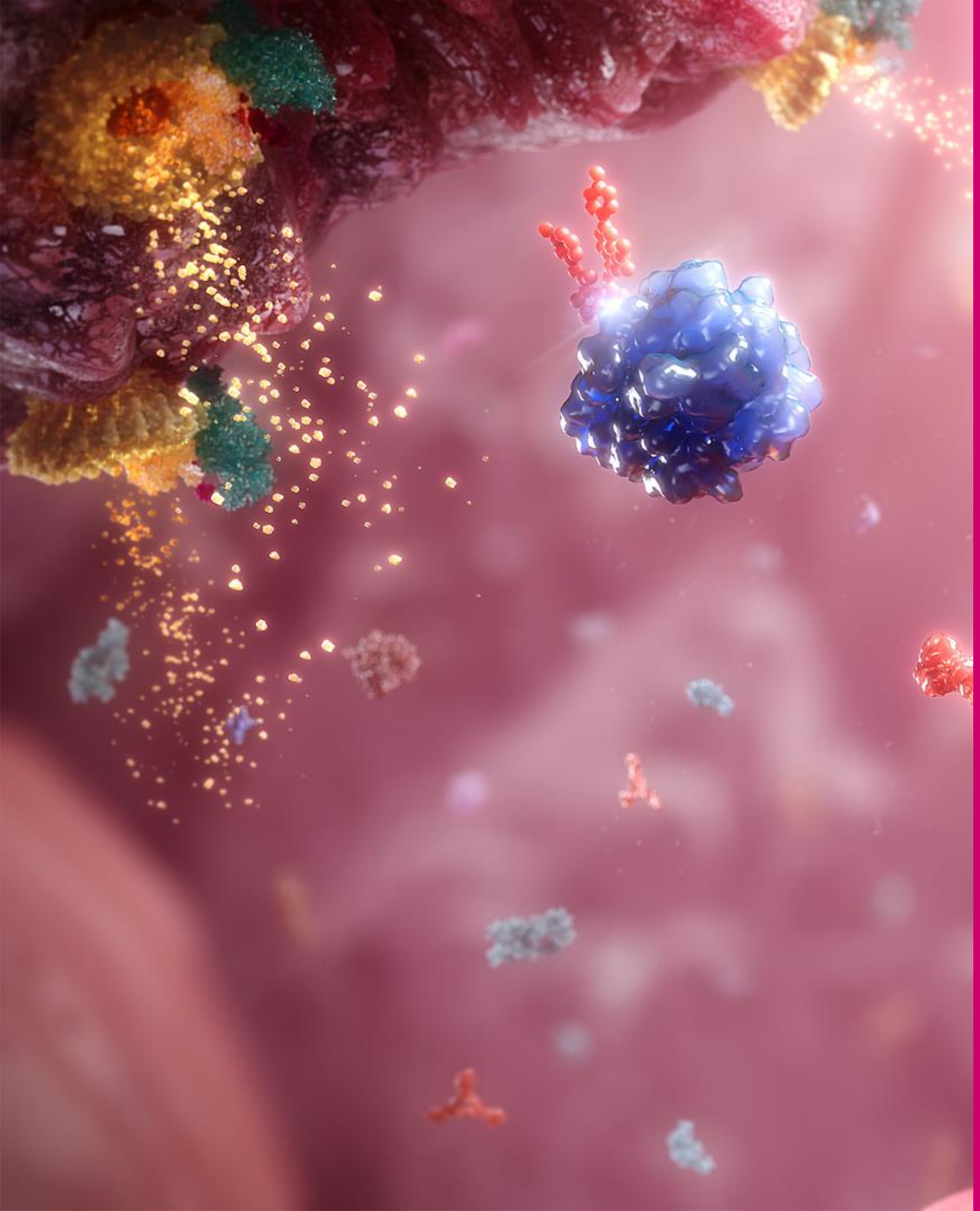
## COVID-19

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**ARTEMIS-C**<br>**NCT06147063** | Healthy volunteers ≥18+ with history of a SARS-CoV-2 infection and/or prior completion of primary series/booster vaccination at least 6 months prior to trial start | 240 | • Arm 1: dose 1 via i.m. injection AZD9838 in 18-64-year-olds<br>• Arm 2: dose 2 via i.m. injection AZD9838 in 18-64-year-olds<br>• Arm 3: i.m. dose of licensed mRNA vaccine in 18-64-year-olds<br>• Arm 4: dose 1 via i.m. injection AZD6563 in 18-64-year-olds<br>• Arm 5: dose 2 via i.m. injection AZD6563 in 18-64-year-olds<br>• Arm 6: dose 1 via i.m. injection in 65+ year olds<br>• Arm 7: dose 2 via i.m. injection in 65+ year olds<br>• Arm 8: i.m dose of licensed mRNA vaccine in 65+ year olds | • Primary endpoints: safety as measured by AEs, ARs, SAEs, MAAEs, AESIs, GMTs of strain neutralising antibodies and GMFRs of strain neutralising antibodies<br>• Secondary endpoints: nAb responses to the SARS-CoV2 ancesteral strain, Omicron BA.4/5, and Omicron XBB.1.5 in serum | • FPCD: Q4 2023<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.



# Rare Disease:
## approved medicines and late-stage development

AstraZeneca

# *Koselugo* (selumetinib, MEK inhibitor)

## Neurofibromatosis type 1, solid tumours

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**KOMET**<br>**NCT04924608** | Adult age ≥18 years with NF1 who have symptomatic, inoperable PN<br><br>Available baseline chronic target PN pain score | 145 | • Multi-centre, international trial with a parallel, randomised, double-blind, placebo-controlled, 2 arm design<br>• Arm 1: *Koselugo* 25mg/m2 BID<br>• Arm 2: placebo BID until end of Cycle 12, then crossover to *Koselugo* 25mg/m2 BID | • Primary endpoint: ORR by end of Cycle 16 on *Koselugo* vs. placebo as determined by ICR per REiNS criteria<br>• Secondary endpoint: change in baseline of chronic PN-pain intensity on *Koselugo* vs. placebo | • FPCD: Q4 2021<br>• Data readout: Q3 2024<br>• Primary endpoint met |
| **Phase I/II**<br>**SPRINKLE**<br>**NCT05309668** | Paediatric (age 1 to 7 years) diagnosed with NF1 with symptomatic, inoperable PN with at least one measurable PN, defined as a PN of at least 3cm, measured in one dimension | 38 | • Single-arm, open-label with *Koselugo* granule formulation | • Primary endpoints: *Koselugo* AUC0-12 derived after single dose administration [time frame: pre-dose and 1, 2, 3, 4, 6, 8 and 10-12 hours after *Koselugo* single dose on the first day of treatment (Cycle 1 Day 1)]; AEs graded by CTCAE Ver 5.0 [time frame: from screening until 30 days after last dose] | • FPCD: Q1 2022<br>• Data readout: Q2 2024<br>• Primary endpoint met |
| **Phase I**<br>**China PK/Safety/Efficacy**<br>**NCT04590235** | Pediatric (age 2 to 17 years old), adult NF1 | 32 | • Single-arm trial with 3 phases: dose confirmation phase (n=6 for 3 cycles), expansion phase (24 months post-LSD) and long-term follow-up (60 months post-LSD) | • Primary endpoints: safety, tolerability and PK parameters<br>• Secondary endpoint: efficacy (ORR, DoR; TTR; PFS) | • FPCD: Q1 2021<br>• Data readout: Q4 2023 |
| **Phase I**<br>**Food Effect/GI Tolerability**<br>**NCT05101148** | Adolescents aged ≥12 to <18 years at trial entry with a clinical diagnosis of NF1-related PN<br><br>Koselugo with a low-fat meal compared to fasted state | 24 | • Single-arm, multiple dose, sequential, two or three period trial<br>• *Koselugo* 25mg/m2 BID given with a low-fat meal vs. the same dose given in a fasted state | • Primary endpoints: PK parameters (steady state systemic exposure), safety (GI toxicity) | • FPCD: Q3 2021<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# *Ultomiris* (anti-C5 mAb)

## Haematology, nephrology

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ALXN1210-TM-313**<br>**NCT04543591** | Thrombotic microangiopathy-associated haematopoietic stem cell transplant | 106 | • Arm 1: *Ultomiris* Q8W<br>• Arm 2: placebo | • Primary endpoint: TMA response<br>• Secondary endpoints: time to TMA response, TMA relapse | • FPCD: Q4 2020<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**ALXN1210-TM-314**<br>**NCT04557735** | Paedeatric thrombotic microangiopathy-associated haematopoietic stem cell transplant | 40 | • Arm 1: *Ultomiris* administered once every 4 to 8 weeks | • Primary endpoint: proportion of participants with TMA response<br>• Secondary endpoints: time to TMA response, proportion of participants with TMA relapse | • FPCD: Q4 2020<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**ARTEMIS**<br>**NCT05746559** | CSA-AKI | 736 | • Randomised, double-blind, placebo-controlled, muticentre trial<br>• *Ultomiris* i.v. to protect patients with CKD from CSA-AKI and subsequent MAKE | • Primary endpoint: to assess the efficacy of a single dose of Ultomiris i.v. vs. placebo in reducing the risk of the clinical consequences of AKI (MAKE) at 90 days in adult participants with CKD who undergo non-emergent cardiac surgery with CPB | • FPCD: Q1 2023<br>• Data anticipated: 2026 |
| **Phase III**<br>**ICAN**<br>**NCT06291376** | Immunoglobulin A nephropathy | 450 | • Arm 1: *Ultomiris* via weight-based i.v. infusion<br>• Arm 2: placebo via weight-based i.v. infusion | • Primary endpoints: change from baseline in proteinuria based on 24-hour UPCR at Week 34 and eGFR over 106 weeks<br>• Secondary endpoints: change from baseline in proteinuria based on 24-hour UPCR at Weeks 10, 26, 34, 50, and 106 and change from baseline in eGFR at Weeks 34, 50, and 106 | • FPCD: Q2 2024<br>• Data anticipated: >2026 |
| **Phase II**<br>**SANCTUARY**<br>**NCT04564339** | Proliferative lupus nephritis or immunoglobulin A nephropathy | 120 | • Arm 1: LN cohort, *Ultomiris*<br>• Arm 2: LN cohort, placebo<br>• Arm 3: IgAN cohort, *Ultomiris*<br>• Arm 4: IgAN cohort, placebo | • Primary endpoint: percentage change in proteinuria from baseline to Week 26<br>• Secondary endpoints: percentage change in proteinuria from baseline to Week 50 | • FPCD: Q1 2021<br>• Data anticipated: 2026<br>• Primary endpoint met (IgAN cohort) |





As of 6 February 2025.
Appendix: Glossary.

# *Ultomiris* (anti-C5 mAb)

## Neurology

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ALXN1210-NMO-307**<br>**NCT04201262** | Neuromyelitis optica spectrum disorder | 58 | • Arm 1: *Ultomiris* Q8W | • Primary endpoint: time to first adjudicated on-trial relapse | • FPCD: Q4 2019<br>• LPCD: Q1 2021<br>• Data readout: Q2 2022<br>• Primary endpoint met |
| **Phase II/III**<br>**ALXN1210-NMO-317**<br>**NCT05346354** | Neuromyelitis optica spectrum disorder | 12 | • Arm 1: *Ultomiris* Q8W | • Primary endpoint: change from baseline in annualised relapse rate at Week 50 | • FPCD: Q3 2022<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# acoramidis (ALXN2060, TTR stabiliser)

## ATTR-CM

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ALXN2060-TAC-302**<br>**NCT04622046** | ATTR-CM | 22 | • Arm 1: 800mg acoramidis administered twice daily<br>• Japan only | • Primary endpoint: change from baseline to Month 12 of treatment in distance walked during the six-minute walk test, cause mortality and cardiovascular related hospitalisation over a 30-month period | • FPCD: Q4 2020<br>• Data readout: Q1 2024<br>• Primary endpoint met |





As of 6 February 2025.
Appendix: Glossary.

# ALXN2220 (NI006, TTR depleter)

## Amyloidosis

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**DepleTTR-CM**<br>**NCT06183931** | ATTR-CM | 1000 | • Arm 1: ALXN2220 via i.v. infusion Q4W for at least 24 months up to a maximum of 48 months<br>• Arm 2: placebo via i.v. infusion Q4W for at least 24 months up to a maximum of 48 months | • Primary endpoints: all-cause mortality and total CV events | • FPCD: Q1 2024<br>• Data anticipated: >2026 |





As of 6 February 2025.
Appendix: Glossary.

# anselamimab (CAEL-101, fibril-reactive mAb)

## AL amyloidosis

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**CAEL101-302**<br>**NCT04512235** | AL amyloidosis<br>(Mayo Stage IIIa) | 267 | • Arm 1: anselamimab combined with SoC for PCD<br>• Arm 2: placebo combined with SoC for PCD | • Primary endpoint: A hierarchical combination of time to all-cause mortality and frequency of cardiovascular hospitalization, safety (TEAEs)<br>• Secondary endpoint: quality of life measures | • FPCD: Q4 2020<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**CAEL101-301**<br>**NCT04504825** | AL amyloidosis<br>(Mayo Stage IIIb) | 124 | • Arm 1: anselamimab combined with SoC for PCD<br>• Arm 2: placebo combined with SoC for PCD | • Primary endpoint: A hierarchical combination of time to all-cause mortality and frequency of cardiovascular hospitalization, safety (TEAEs)<br>• Secondary endpoint: quality of life measures | • FPCD: Q1 2021<br>• Data anticipated: H2 2025 |
| **Phase II**<br>**CAEL101-203**<br>**NCT04304144** | AL amyloidosis (Mayo Stage I, Stage II and Stage IIIa) | 25 | • Arm 1: anselamimab combined with SoC CyBorD<br>• Arm 2: placebo combined with SoC CyBorD and daratumumab | • Primary endpoint: occurrence of DLT during the first 4 weeks of therapy<br>• Secondary endpoint: AUC (plasma curve concentration) | • FPCD: Q1 2020<br>• Data readout: Q2 2024 |





As of 6 February 2025.
Appendix: Glossary.

# efzimfotase alfa (ALXN1850, next-generation asfotase alfa)

## Hypophosphatasia

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**ALXN1850-HPP-101**<br>**NCT04980248** | Hypophosphatasia | 15 | • Arm 1: ALXN1850, 3 cohorts at low, medium and high dosages | • Primary endpoint: incidence of TEAEs and TESAEs | • FPCD: Q3 2021<br>• Data readout: Q4 2022<br>• Primary endpoint met |
| **Phase III**<br>**HICKORY**<br>**NCT06079281** | Hypophosphatasia | 114 | • Arm 1: placebo on Day 1 followed by Q2W via s.c. injection for 24 weeks<br>• Arm 2: bodyweight-dependent doses of either 20mg, 35mg or 50mg of efzimfotase alfa Q2W via s.c. injection for 24 weeks | • Primary endpoint: change from baseline in 6MWT at Day 169 | • FPCD: Q2 2024<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**CHESTNUT**<br>**NCT06079372** | Hypophosphatasia | 40 | • Arm 1: bodyweight-dependent doses of either 20mg, 35mg or 50mg of efzimfotase alfa Q2W via s.c. for 24 weeks<br>• Arm 2: 6mg/kg/week of Strensiq via s.c. injection as either 2mg/kg 3 times per week or 1mg/kg 6 times per week for 24 weeks | • Primary endpoint: number of participants TEAEs | • FPCD: Q2 2024<br>• Data anticipated: H2 2025 |
| **Phase III**<br>**MULBERRY**<br>**NCT06079359** | Hypophosphatasia | 30 | • Arm 1: bodyweight-dependent doses of either 25mg, 35mg, or 50mg of efzimfotase Q2W via s.c. injection for 24 weeks<br>• Arm 2: placebo Q2W for 24 weeks | • Primary endpoint: Radiographic Global Impression of Change (RGI-C) Score at Day 169 | • FPCD: Q3 2024<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# eneboparatide (parathyroid hormone receptor 1 agonist)

## Hypoparathyroidism

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**CALYPSO**<br>**NCT05778071** | Chronic hypoparathyroidism | 165 | • Arm 1: 20mcg eneboparatide administered once daily via s.c. injection<br>• Arm 2: placebo administered once daily via s.c. injection | • Primary endpoint: complete independence from active vitamin D, independence from therapeutic doses of oral calcium (i.e. taking oral elemental calcium supplements ≤600mg/day) and albumin-adjusted serum calcium within the normal range (8.3 to 10.6mg/dL) vs. placebo after 24 weeks of treatment | • FPCD: Q3 2023<br>• Data anticipated: H1 2025 |





As of 6 February 2025.
Appendix: Glossary.

# gefurulimab (ALXN1720, anti-C5 humanised bispecific heavy-chain antibody)

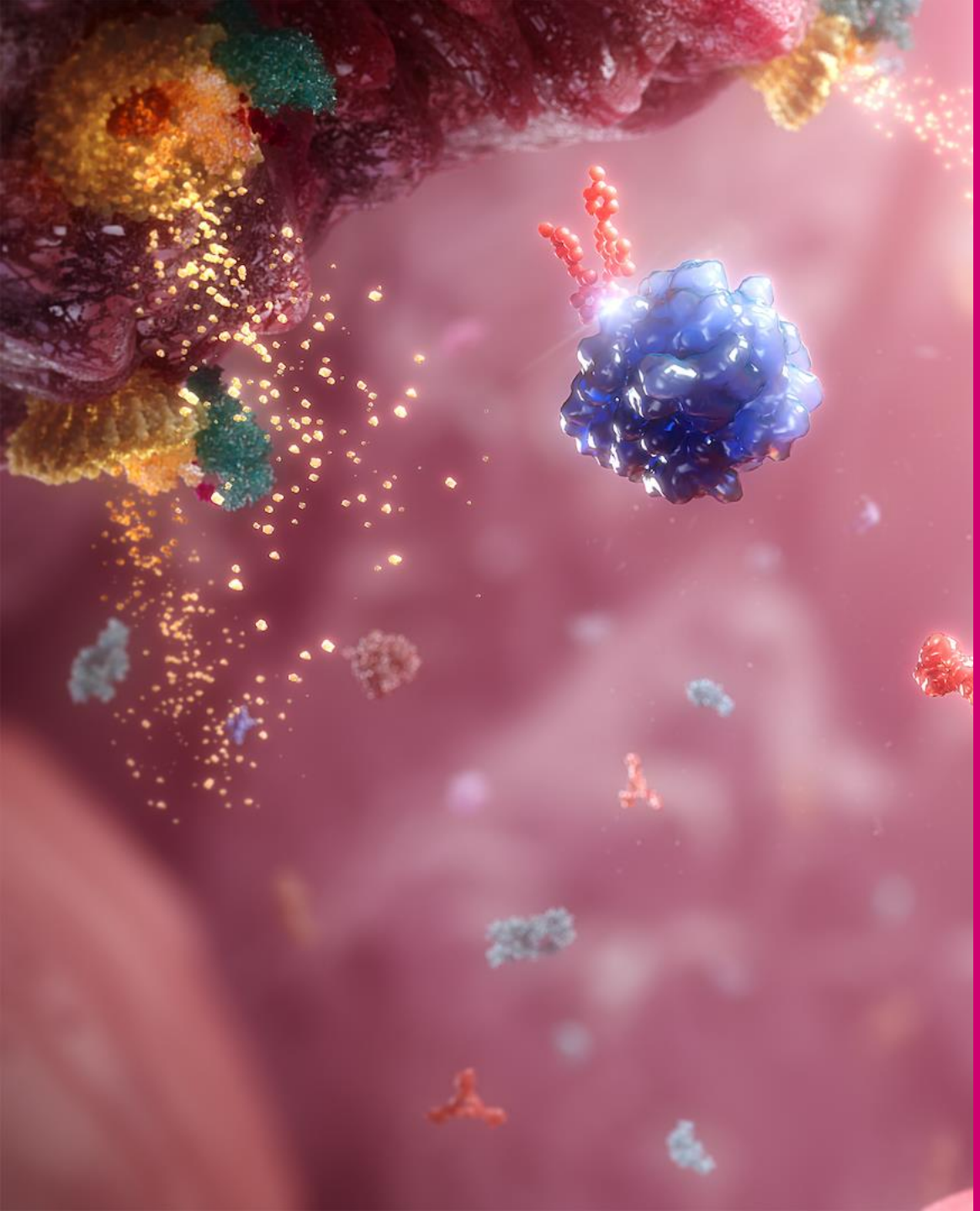
## Neurology, nephrology

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase III**<br>**ALXN1720-MG-301**<br>**NCT05556096** | Generalised myasthenia gravis | 254 | • Arm 1: weight-based maintenance treatment with gefurulimab on Day 1, followed by weight-based maintenance treatment of gefurulimab on Week 1 (Day 8) and Q1W thereafter for a total of 26 weeks<br>• Arm 2: placebo | • Primary endpoint: change from baseline in MG-ADL total score at Week 26 | • FPCD: Q4 2022<br>• Data anticipated: H2 2025 |
| **Phase I**<br>**ALXN1720-NEPH-102**<br>**NCT05314231** | Proteinuria | 13 | • Arm 1: gefurulimab s.c. infusion at a dose of 1500mg | • Primary endpoint: serum concentration of [time frame: Day 1 (0.5 hours pre-dose and post-dose) and dose on Days 2, 3, 8, 15, 29, 43 and 57] | • FPCD: Q2 2022<br>• Data readout: Q3 2023 |





As of 6 February 2025.
Appendix: Glossary.



# Rare Disease:
## early-stage development

AstraZeneca

# ALXN1910 (next-generation TNSALP ERT)

## Bone metabolism

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**ALXN1910-HV-101**<br>**NCT05307978** | Healthy adults | 48 | • Randomised, placebo-controlled SAD trial | • Primary endpoint: safety | • FPCD: Q2 2022<br>• Data readout: Q2 2023<br>• Trial discontinued due to efficacy |





As of 6 February 2025.
Appendix: Glossary.

# ALXN1920 (kidney-targeted factor H fusion protein)

## Nephrology

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**ALXN1920-HV-101**<br>**NCT05751642** | Healthy adults | 48 | • Randomised, double-blind, placebo-controlled, SAD trial | • Primary endpoints: safety and tolerability<br>• Secondary endpoints: PK/PD parameters | • FPCD: Q2 2023<br>• Data readout: Q2 2024 |





As of 6 February 2025.
Appendix: Glossary.

# ALXN2030 (siRNA targeting complement C3)

## Nephrology

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**ALXN2030-HV-101**<br>**NCT05501717** | Healthy volunteers | 48 | • Randomised, placebo-controlled SAD trial | • Primary endpoint: safety | • FPCD: Q4 2022<br>• Data anticipated: 2026 |





As of 6 February 2025.
Appendix: Glossary.

# ALXN2080 (factor D inhibitor)

## Complement-mediated disease

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase I**<br>**ALXN2080-HV-101**<br>**NCT05428696** | Healthy volunteers | 90 | • SAD/MAD trial | • Primary endpoints: safety and tolerability, PK and PD parameters | • FPCD: Q3 2022<br>• Data readout: Q3 2023 |





As of 6 February 2025.
Appendix: Glossary.

# MEDI1341 (alpha-synuclein mAb)

## Multiple system atrophy

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**NCT05526391**<br>**Partnered (Takeda)** | Patients with diagnosis of possible or probably MSA (using modified Gilman et al. 2008 diagnostic criteria) | 138 | • Randomised, double-blind, placebo-controlled trial<br>• Early PK cohort<br>• Arm 1: TAK-341/MEDI1341 i.v.<br>• Arm 2: placebo i.v.<br>• Main cohort<br>• Arm 3: TAK-341/MEDI1341 i.v.<br>• Arm 4: placebo i.v. | • Primary endpoints: efficacy, change from baseline on modified Unified Multiple System Atrophy Rating Scale at 52 weeks<br>• Secondary endpoints: PK parameters, safety and efficacy | • FPCD: Q4 2022<br>• Data anticipated: H2 2025 |





As of 6 February 2025.
Appendix: Glossary.

# vemircopan (ALXN2050, factor D inhibitor)

## Haematology, nephrology, neurology

| Trial | Population | Patients | Design | Endpoints | Status |
|---|---|---|---|---|---|
| **Phase II**<br>**ALXN2050-NEPH-201**<br>**NCT05097989** | Lupus nephritis or immunoglobulin A nephropathy | 126 | • Arm 1 – LN cohort: vemircopan 180mg<br>• Arm 2 – LN cohort: vemircopan 120mg<br>• Arm 3 – LN cohort: placebo<br>• Arm 4 – IgAN cohort: vemircopan 180mg<br>• Arm 5 – IgAN cohort: vemircopan 120mg<br>• Arm 6 – IgAN cohort: placebo | • Primary endpoint (both cohorts): percentage change in proteinuria from baseline to Week 26 | • FPCD: Q3 2022<br>• Data anticipated: 2026<br>• Trial discontinued due to lack of efficacy |
| **Phase I**<br>**ALXN2050-HV-109**<br>**NCT05259085** | Impaired hepatic function | 36 | • Arm 1: mild IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4<br>• Arm 2: moderate IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4<br>• Arm 3: severe IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4<br>• Arm 4: healthy control, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 | • Primary endpoint (Arm 1): AUC0-12 of plasma vemircopan after steady-state<br>• Primary endpoint (Arm 2): AUCt of plasma vemircopan after steady-state<br>• Primary endpoint (Arm 3): Cmax,ss of vemircopan<br>• Primary endpoint (Arm 4): Tmax,ss following vemircopan | • FPCD: Q2 2022<br>• Data anticipated: H1 2025<br>• Trial discontinued due to lack of efficacy |



As of 6 February 2025.
Appendix: Glossary.

NEXT

# Glossary – 1 of 5

| | |
|---|---|
| 14C | Carbon 14 |
| 1L, 2L, 3L | 1st-, 2nd- or 3rd-line |
| 5-FU | 5-fluorouracil |
| 6MWT | 6-minute walk test |
| A2AR | Adenosine A2A receptor |
| AAV | Adeno-associated virus |
| ACE | Angiotensin-converting enzyme |
| AChR+ | Acetylcholine receptor-positive |
| ACQ | Asthma Control Questionnaire |
| ACR | American College of Rheumatology Response Scoring System |
| ADA | Anti-drug antibody |
| ADC | Antibody-drug conjugate |
| ADP | Adenosine diphosphate |
| ADsCa | Albumin-adjusted serum calcium |
| AE | Adverse event |
| AER | Annual exacerbation rate |
| AEs | Adverse effects |
| AGA | Actional genomic alteration |
| aHUS | Atypical haemolytic uraemic syndrome |
| AI | Auto-injector |
| AI | Aromatase inhibitor |
| AKT | Protein kinase B |
| AL amyloidosis | Light-chain amyloidosis |
| ALK | Anaplastic large-cell lymphoma kinase |
| ALL | Acute lymphocytic leukaemia |
| alloSCT | Allogeneic stem cell tranplantation |
| ALSFRS-R | Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised |
| AML | Acute myeloid leukaemia |
| AMR | Antibody mediated rejection |
| anti-FRα | Anti-folate receptor alpha |
| anti-PCD | Anti-plasma cell dyscrasia |
| APFS | Accessorised pre-filled syringe |
| APOL1 | Apolipoprotein L1 |
| APOL1 G0/G1/G2 | Sequences of the G0, G1, and G2 APOL1 variants from amino acids 339–398 |
| AQLQ | Asthma Quality of Life Questionnaire |
| AQP4+ | Aquaporin-4 antibody positive |
| ARB | Angiotensin receptor blockers |
| AS | Albuterol sulfate |
| ASCO | American Society of Clinical Oncology |
| ASI | Aldosterone synthase inhibitor |
| ASO | Antisense oligonucleotide |
| ATM | Ataxia telangiectasia mutated kinase |
| ATR | Ataxia telangiectasia and Rad3-related protein |
| ATTR | Transthyretin amyloidosis |
| ATTR-CM | Transthyretin amyloid cardiomyopathy |
| ATTR-PN | Transthyretin amyloid polyneuropathy |
| ATTRv-PN | Hereditary transthyretin-mediated amyloid polyneuropathy |
| AUC | Area under curve |
| AUCinf | Area under plasma concentration time curve from zero to infinity |
| AUClast | Area under plasma concentration curve from zero to the last quantifiable concentration |
| AUCt | Area under concentration-time curve |
| AUEC | Area under the effect-time curve |
| Avb8 | Alpha v beta 8 |
| B7H4 | B7 homolog 4 |
| BA | Bioavailability |
| BAFF | B-cell activating factor |
| B-ALL | B cell acute lymphoblastic leukaemia |
| BBB | Blood-brain barrier |
| BCG | Bacillus Calmette-Guérin |
| BCL2 | B-cell leukemia/lymphoma 2 protein |
| BCMA | B-cell maturation antigen |
| BDA | Budesonide albuterol |
| BFF | Budesonide and formoterol fumarate |
| BGF | Budesonide, glycopyrronium and formoterol fumarate |
| BICLA | British Isles Lupus Assessment Group-based Composite Lupus Assessment |
| BICR | Blinded independent central review |
| BID | Twice per day |
| BIG | Big Ten Cancer Research Consortium |
| BM | Biomarker |
| BMD | Bone mineral density |
| BMFI | Bone metastasis-free interval |
| BMI | Body mass index |
| BOR | Best overall response rate |
| BR | Bendamustine and rituximab |
| BRCA | BReast CAncer gene |
| BRCAm | BReast CAncer gene-mutated |
| BRCAwt | BReast CAncer wild-type gene |
| BRD4 | Bromodomain-containing protein 4 |
| BTC | Biliary tract carcinoma |
| BTC | Biliary tract cancer |
| BTK | Bruton's tyrosine kinase |
| BTKi | Bruton's tyrosine kinase |
| BVAS | Birmingham Vasculitis Activity Score |
| C3 | Complement component 3 |
| C5 | Complement component 5 |
| CA-125 | Cancer antigen-125 |
| CAAT | Chronic Airways Assessment Test |
| CAD | Coronary artery disease |
| CAGR | Compound annual growth rate |
| cAMR | Chronic antibody-medicated rejection |
| CAR-T | Chimeric antigen receptor therapy |
| CBP | Cardiopulmonary bypass |
| CBR | Clinical benefit rate |
| CD | Cluster of differentiation |
| CD123 | Interleukin 3 receptor a |
| CD19 | Cluster of differentiation 19 |
| CD3 | Cluster of differentiation 3 |
| CD39 | Cluster of differentiation 39 |
| CD73 | Cluster of differentiation 73 |
| CD8 | Cluster of differentiation 8 |
| CDAI | Clinical Disease Activity Index |
| CDK | Cyclin-dependent kinase |
| CDK2 | Cyclin-dependent kinase 2 |
| CDK4/6i | Cyclin-dependent kinase 4/6 inhibitor |
| CE | Clinically evaluable |
| CHD | Coronary heart disease |
| Chemo | Chemotherapy |
| CHF | Chronic heart failure |
| cHL | Classic Hodgkin lymphoma |
| CI | Confidence interval |
| CKD | Chronic kidney disease |
| CLD | Chronic lung disease |
| CLDN 18.2 | Claudin-18.2 |
| CLDN18.2 | Claudin 18.2 |
| CLL | Chronic lymphocytic leukaemia |
| cm | Centimetre |
| CM | Cardiomyopathy |
| CMAX | Maximum observed plasma concentration |
| cMET | C-mesenchymal epithelial transition factor |
| CMML | Chronic myelomonocytic leukaemia |



As of 6 February 2025.

Appendix: Glossary.

# Glossary – 2 of 5

| | |
|---|---|
| CNS | Central nervous system |
| CNS-PFS | Central nervous system progression-free survial |
| CompEx | Composite endpoint for exacerbations |
| COPD | Chronic obstructive pulmonary disease |
| CPB | Cardiopulmonary bypass |
| CPI | Checkpoint inhibitor |
| CPI-experienced | Checkpoint inhibitor-experienced |
| CPI-naive | Checkpoint inhibitor-naïve |
| cPR | Central pathological review |
| CR | Complete response |
| CRC | Colorectal cancer |
| CrCl | Creatinine clearance |
| CRR | Complete response rate |
| CRR | Complete renal response |
| CRSwNP | Chronic rhinosinusitis with nasal polyps |
| CRT | Chemoradiotherapy |
| CRwNP | Chronic rhinosinusitis with nasal polyps |
| CSA-AKI | Cardiac surgery-associated acute kidney injury |
| CTC | Circulating tumour cell |
| CTCAE | Common Terminology Criteria for Adverse Events |
| ctDNA | Circulating tumour DNA |
| CTLA4 | Cytotoxic T-lymphocyte associated protein 4 |
| CTLA-4 | Cytotoxic T-lymphocyte-associated antigen-4 |
| CTx | Chemotherapy |
| CV | Cardiovascular |
| CVOT | Cardiovascular outcomes trial |
| CVRM | Cardiovascular, Renal and Metabolism |
| CXCR2 | C-X-C Motif chemokine receptor 2 |
| CyBorD | Cyclophosphamide, bortezomib and dexamethasone |
| Datroway | Datroway |
| DCR | Disease control rate |
| DDFS | Distant disease-free survival |
| DDI | Drug-drug Interaction |
| DDR | DNA damage response |
| dECG | Differentiated electrocardiogram |
| DFS | Disease-free survival |
| DGF | Delayed graft function |
| DLBCL | Diffuse large B-cell lymphoma |
| DLT | Dose-limiting toxicity |
| DMARDs | Disease-modifying antirheumatic drugs |
| DNA | Deoxyribonucleic acid |
| dNCC | Directly measured non-ceruloplasmin-bound copper |
| dnTGFb | Dominant-negative transforming growth factor-beta |
| DoCR | Durability of complete response |
| DoR | Duration of response |
| DPB | Disease progression in bone |
| DPI | Dry powder inhaler |
| dPTEN | Phosphatase and tensin homolog deficient |
| DRFI | Disease recurrence-free interval |
| DSQ | Dysphagia Symptom Questionnaire |
| DXA | Dual energy X-ray absorptiometry |
| EBITDA | Earnings before interest, tax, depreciation and amortisation |
| EBRT | External beam radiation therapy |
| ECG | Electrocardiogram |
| ED | Emergency department |
| EFS | Event-free survival |
| EG | Eosinophilic gastritis |
| EGE | Eosinophilic gastroenteritis |
| eGFR | Estimated glomerular filtration rate |
| eGFR | Epidermal growth factor receptor-mutated |
| EGFRi | Epidermal growth factor receptor inhibitor |
| EGFRm | Epidermal growth factor receptor-mutated |
| EGPA | Eosinophilic granulomatosis with polyangiitis |
| EM | Emerging Markets |
| EoE | Eosinophilic oesophagitis |
| EOS | Eosinophil |
| EPI | Epigenetics |
| ER | Estrogen receptor |
| ER+ | Estrogen receptor-positive |
| ERK | Extracellular signal-regulated kinase |
| ERoW | Established Rest of World |
| E-RS:COPD | Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease |
| ERT | Enzyme replacement therapy |
| ESAI | Eczema Area and Severity Index |
| ESCC | Esophageal squamous cell carcinoma |
| ESKD | Early-stage kidney disease |
| ESR1 | Estrogen receptor 1 |
| ESRD | End-stage renal disease |
| ET | Endocrine therapy |
| ETA | Endothelin A |
| ETA | Endothelin A |
| ETA RA | Endothelin receptor A antagonist |
| EU | European Union |
| EVH | Extravascular haemolysis |
| FAF | Fundus autofluorescence |
| FCR | Fludarabine, cyclophosphamide and rituximab |
| FDC | Fixed-dose combination |
| FeNO | Fractional nitric oxide concentration in exhaled breath |
| FEV | Forced-expiratory volume |
| FEV1 | Forced expiratory volume in 1 second |
| FGFR | Fibroblast growth factor receptor |
| FL | Follicular lymphoma |
| FLAP | 5-lipoxygenase activating protein |
| FLOT | Fluorouracil, leucovorin, oxaliplatin and docetaxel |
| FOLFOX | Folinic acid, fluorouracil and oxaliplatin |
| FOXP3 | Forkhead box P3 |
| FP | 5-fluorouracil/cisplatin |
| FPCD | First patient commenced dosing |
| FPG | Fasting plasma glucose |
| FRα | Folate receptor alpha |
| FX | Foreign exchange |
| G7 | US, Japan, EU5 |
| GA | Geographic atrophy |
| GBM | Glioblastoma |
| gBRCAm | Germline BRCA-mutated |
| GC | Gastric cancer |
| GCB | Germinal center B-cell |
| GEJ | Gastric/gastroesophogeal junction |
| GEJC | Gastroesophageal junction cancer |
| GFF | Glycopyrronium and formoterol fumarate |
| GI | Gastrointestinal |
| GLP-1 | Glucagon-like peptide-1 |
| GLP-1/glu | Glucagon-like peptide 1 receptor/glucagon dual peptide agonist |
| GLP-1RA | Glucagon-like peptide 1 receptor agonist |
| GMFR | Geometric mean fold rise |
| gMG | Generalised myasthenia gravis |
| GMT | Geometric mean titer |
| GN | Glomerulonephritis |
| GPC3 | Glypican-3 |
| GPC3-positive | Glypican 3-positive |



As of 6 February 2025.
Appendix: Glossary.

# Glossary – 3 of 5

| | |
|---|---|
| GPRC5D | G protein-coupled receptor, class C, group 5, member D |
| GU | Genitourinary |
| GYN | Gynaecologic |
| H1 | H1-antihistamine |
| hADME | Human mass balance |
| HbA1c | Glycated haemoglobin |
| HCC | Hepatocellular carcinoma |
| HD | High dose |
| HDL-C | High-density lipoprotein cholesterol |
| HER2 | Human epidermal growth factor receptor 2 |
| HER2-low | Human epidermal growth factor receptor 2-low |
| HER2-negative | Human epidermal growth factor receptor 2-negative |
| HER2-positive | Human epidermal growth factor receptor 2-positive |
| HES | Hyper eosinophilic syndrome |
| HF | Heart failure |
| HFA | Hydrofluoroalkane |
| HFO | Hydrofluoro-olefins |
| HFpEF | Heart failure with preserved ejection fraction |
| HFrEF | Heart failure with reduced ejection fraction |
| HGFR | Met/hepatocyte growth factor receptor |
| HGSC | High-grade serous carcinoma |
| hHF | Hospitalisation for heart failure |
| HIF-PH | Hypoxia inducible factor-prolyl hydroxylase |
| HK | Hyperkalaemia |
| HLA-A*02:01 | Human leukocyte antigen serotype within the HLA-A serotype group |
| HLR | High-level results |
| hMPV | Human metapneumovirus |
| HNSCC | Head and neck squamous-cell carcinoma |
| HPD | Hyperprogressive disease |
| HPDD | Highest protocol-defined dose |
| HPF | High-power field |
| HPP | Hypophosphatasia |
| HR | Hazard ratio |
| HR+ | Hormone receptor-positive |
| HRD | Homologous recombination deficiency |
| HRD+ | Homologous recombination deficiency-positive |
| HR-low | Hormone receptor-low |
| HRR | homologous recombination repair |
| HRRm | Homologous recombination repair-mutated |
| HSCT-TMA | hematopoietic stem cell transplantation-associated thrombotic microangiopathy |
| HSD17B13 | Hydroxysteroid 17-beta dehydrogenase 13 |
| HVPG | Hepatic venous pressure gradient |
| i | Inhibitor |
| i.m. | Intramuscular |
| i.v. | Intravenous |
| IA | Investigator-assessed |
| IBD | Inflammatory bowel disease |
| ICR | Independent central review |
| ICS | Inhaled corticosteroid |
| ICS-LABA | Inhaled corticosteroid long-acting beta-agonists |
| ICU | Intensive care unit |
| IDFS | Invasive disease-free survival |
| IgAN | Immunoglobulin A nephropathy |
| IHF | Impaired hepatic function |
| IIT | Investigated initiated trial |
| iJAK1 | Inhaled Janus kinase |
| IL | Interleukin |
| IL-12 | Interleukin-12 |
| IL-33 | Interleukin-33 |
| IL-5 | Interleukin-5 |
| IL-5R | Interleukin-5 receptor |
| IMAC-TIS | International Myositis Assessment And Clinical Studies-Total Improvement Score |
| IND | Investigational new drug |
| INV | Investigator review |
| IO | Immuno-oncology |
| IPF | Idiopathic pulmonary fibrosis |
| IPFS | Invasive progression-free survival |
| IRA | Inflation Reduction Act |
| IRAK4 | Interleukin-1 receptor-associated kinase 4 |
| IRC | Independent review committee |
| ISS | Investigator-sponsored studies |
| ISS7 | Itch-severity score (weekly) |
| iTSLP | Inhaled thymic stromal lymphopoietin |
| ITT | Intent-to-treat |
| IVIg | Intravenous immunoglobulin |
| JAK-1 | Janus kinase 1 |
| K+ | Potassium |
| KCCQ | Kansas City Cardiomyopathy Questionnaire |
| kg | Kilogram |
| Ki67 | Antigen Kiel 67 |
| LA amylin | Long-acting amylin |
| LAAB | Long-acting antibody |
| LABA | Long-acting beta agonist |
| LAMA | Long-acting muscarinic agonist |
| LCAT | Lecithin-cholesterol acyltransferase |
| LCM | Lifecycle management |
| LDH | Lactate dehydrogenase |
| LDL-C | Low-density lipoprotein cholesterol |
| LICA | Ligand-conjugated ASO |
| LIF | Low-density lipoprotein cholesterol |
| LN | Lupus nephritis |
| LoE | Loss of exclusivity |
| LOS | Length of stay |
| LPCD | Last patient commenced dosing |
| LSD | Last subject dosed |
| LS-SCLC | Limited stage small-cell lung cancer |
| LV | Left ventricle |
| m | Mutation |
| mAb | Monoclonal antibody |
| MABA | Muscarinic antagonist-beta2 agonist |
| MACE | Major adverse cardiac events |
| MAD | Multiple ascending dose |
| MAKE | Major adverse kidney events |
| MASH | Metabolic dysfunction-associated steatohepatitis |
| MASLD | Metabolic dysfunction-associated steatotic liver disease |
| mBC | Metastatic breast cancer |
| MCC | Mucociliary clearance |
| MCL | Mantle cell lymphona |
| mCRPC | Metastatic castrate-resistant prostate cancer |
| MDI | Metered-dose inhaler |
| mDOR | Median duration of response |
| MDS | Myelodysplastic syndrome |
| MEK | Mitogen-activated protein kinase |
| MET | Mesenchymal epithelial transition factor |
| mFOLFOX | Modified folinic acid, fluorouracil and oxaliplatin |
| mg | Milligram |
| mg/dL | Milligrams per decilitre |
| MG-ADL | Myasthenia Gravis-Activities of Daily Living |
| MGFA | Myasthenia Gravis Foundation of America |
| mHSPC | Metastatic hormone sensitive prostate cancer |



As of 6 February 2025.
Appendix: Glossary.

# Glossary – 4 of 5

| | |
|---|---|
| MI | Myocardial infarction |
| mL | Millilitre |
| MM | Multiple myeloma |
| MMAE | Monomethyl auristatin E |
| MMT | Mixed meal test |
| MoA | Mechanism of action |
| mPFS | Median progression-free survival |
| MPO | Myeloperoxidase |
| mPR | Major pathological response |
| MR | Mineralocorticoid receptor |
| MRA | Mineralocorticoid receptor antagonist |
| MRD-negative | Minimal residual disease-negative |
| MRI | Magnetic resonance imaging |
| MRM | Mineralocorticoid receptor modulator |
| mRNA | Messenger ribonucleic acid |
| MSA | Multiple system atrophy |
| MTAP-deficient | Methylthioadenosine phosphorylase-deficient |
| MTD | Maximum tolerated dose |
| mTNBC | Metastatic triple-negative breast cancer |
| MZL | Marginal zone lymphoma |
| n/m | Not material |
| nAb | Neutralising antibody |
| NaC | Sodium channel |
| NAFLD | Non-alcoholic fatty liver disease |
| NASH | Non-alcoholic fatty liver disease |
| NBRx | New-to-brand prescription |
| NCFB | Non-cystic fibrosis bronchiectasis |
| NCI | National Cancer Institute |
| NCPV | Noncalcified plaque volume |
| Neo-adj | Neoadjuvant |
| NF1 | Neurofibromatosis type 1 |
| NF1-PN | Neurofibromatosis type 1 with plexiform neurofibromas |
| ng | Next-generation |
| NGF | Nerve growth factor |
| ngSERD | Next-generation oral selective estrogen receptor degrader |
| NHA | Novel hormonal agent |
| NHL | Non-Hodgkin's lymphoma |
| NIH | National Institute of Health |
| NKTCL | Extranodal natural killer T-cell lymphoma |
| NME | New molecular entity |
| NME | New molecular entity |
| NMOSD | Neuromyelitis optica spectrum disorder |
| NP | Nasal polyps |
| NRDL | National Reimbursement Drug List |
| NRG | National Clinical Trials Network in Oncology |
| NSCLC | Non-small cell lung cancer |
| NST | Neoadjuvant systemic treatment |
| NT-proBNP | N-terminal pro-B-type natriuretic peptide |
| NYHA | New York Heart Association |
| OBD | Optimal biological dose |
| OCS | Oral corticosteroid |
| OD | Once daily |
| oGLP1 | Oral glucagon-like receptor peptide 1 |
| OGTT | Oral glucose tolerance test |
| oPCSK9 | Oral protein convertase subtilisin/kexin type 9 |
| OR | Objective response |
| ORR | Overall response rate |
| oRXFP1 | Oral relaxin family peptide receptor 1 |
| OS | Overall survival |
| PA | Primary aldosteronism |
| PALB2m | Partner and localizer of BRCA2-mutated |
| PAR2 | Protease-activated receptor 2 |
| PARP | Poly ADP ribose polymerase |
| PARP1 | poly(ADP-ribose) polymerase-1 |
| PARP-1sel | Poly ADP ribose polymerase-1 selective |
| PARPi | poly-ADP ribose polymerase inhibitor |
| PASI | Psoriasis area severity index |
| PBD | Pyrrolobenzodiazepine |
| PCD | Plasma cell dyscrasia |
| pCR | Pathological complete response |
| PCSK9 | Proprotein convertase subtilisin/kexin type 9 |
| PD | Pharmacodynamics |
| PD1 | Programmed cell death protein 1 |
| PD-1 | Programmed cell death protein-1 |
| PDAC | Pancreatic ductal adenocarcinoma |
| PDE4 | Phosphodiesterase type 4 |
| PD-L1 | Programmed death-ligand 1 |
| PD-L1-high | Programmed death-ligand 1-high |
| Peak | Maximum |
| PET | Positron-emission tomography |
| PFS | Progression-free survival |
| PFS2 | Time to second disease progression or death |
| PgR | Progesterone receptor |
| PI3K | Phosphoinositide 3 kinase |
| PIK3CA | Phosphatidylinositol-4,5-biphosphate 3-kinase catalytic subunit |
| PK | Pharmacokinetic |
| PK/PD | Pharmacokinetic/pharmacodynamic |
| PLEX | Plasma exchange |
| PLL | Prolymphocytic leukaemia |
| pMDI | Pressurised metered-dose inhaler |
| PN | Plexiform neurofibroma |
| PN | Polyneuropathy |
| PNH | Paroxysmal nocturnal haemoglobinuria |
| PNH-EVH | PNH with extravascular haemolysis |
| PNPLA3 | Phospholipase domain-containing protein 3 |
| POC | Proof-of-concept |
| POM | Proof-of-mechanism |
| post-BD | Post-bronchodilator |
| PP | Plasmapheresis |
| pPCI | Primary percutaneous coronary intervention |
| PR | Partial response |
| pre-BD | Pre-bronchodilator |
| PRMT5 | Protein arginine methyltransferase 5 |
| PRO | Patient reported outcome |
| PRR | Recurrent platinum resistant |
| PS | Propensity score |
| PSA | Prostate-specific antigen |
| PSA50 | Prostate-specific antigen 50 |
| PSC | Pulmonary sarcomatoid carcinoma |
| PSMA | Prostate-specific membrane antigen |
| PSR | Platinum-sensitive relapsed |
| PTCL | Peripheral T-cell lymphoma |
| PTEN | Phosphatase and tensin homolog gene |
| PTH | parathyroid hormone receptor |
| PVR | Pulmonary vascular resistance |
| Q1W | Every one week |
| Q2W | Every two weeks |
| Q4W | Every four weeks |
| Q8W | Every eight weeks |
| QCS | Quantitative continuous scoring |



As of 6 February 2025.

Appendix: Glossary.

# Glossary – 5 of 5

| | |
|---|---|
| QD | Once daily |
| QID | Four times per day |
| QOD | Every other day |
| QoL | Quality of life |
| QoL-DN | Norfolk Quality of Life-Diabetic Neuropathy |
| QT | Duration of ventricular electrical systole |
| QTcF | Corrected QT interval by Fredericia |
| R&I | Respiratory and Immunology |
| R/R | Relapsed/refractory |
| r/r | Relapsed/refractory |
| RA | Rheumatoid arthritis |
| RAAS | Renin-angiotensin-aldosterone system |
| RAGE | Receptor for advanced glycation end products |
| RC | Radioconjugates |
| RECIST | Response Evaluation Criteria in Solid Tumours |
| REiNS | Response Evaluation in Neurofibromatosis and Schwannomatosis |
| RET | Rearranged during transfection |
| RFS | Relapse-free survival |
| rhLCAT | Recombinant human lecithin-cholesterol acyltransferase |
| rNDV | Recombinant Newcastle disease virus |
| RORγ | Related orphan receptor gamma |
| RP2D | Recommended Phase II dose |
| rPFS | Radiographic progression-free survival |
| RR | Response rate |
| RSV | Respiratory syncytial virus |
| RT | Radiation therapy |
| s. asthma | Severe asthma |
| s.c. | Subcutaneous |
| SABA | Short-acting beta2-agonist |
| SAD | Single ascending dose |
| SAE | Serious adverse event |
| SARS-CoV-2 | Severe-acute-respiratory-syndrome-related coronavirus-19 |
| SBP | Systolic blood pressure |
| SBRT | Stereotactic body radiation therapy |
| SCCHN | Squamous-cell carcinoma of the head and neck |
| SCD | Sickle cell disease |
| SCLC | Small cell lung cancer |
| SD | Stable disease |
| SERD | Selective estrogen receptor degrader |
| SG&A | Selling, General and Administrative |
| SGLT2 | Sodium-glucose transport protein 2 |
| SGLT2i | Sodium/glucose cotransporter 2 inhibitor |
| SGRM | Selective glucocorticoid receptor modulator |
| SGRQ | Saint George Respiratory Questionnaire |
| siRNA | Small interfering ribonucleic acid |
| SJC | Swollen joint count |
| sK | Serum potassium |
| SLE | Systemic lupus erythematosus |
| SLL | Small lymphocytic lymphoma |
| SMAD | Single and multiple ascending dose trial |
| SoC | Standard-of-care |
| sPGA | Static Physician's Global Assessment Score |
| SS | Steady state |
| ST2 | Suppression of tumourigenicity 2 |
| STAT3 | Signal transducer and activator of transcription 3 |
| Stg. I/II/III | Stage I/II/III |
| sUA | Serum uric acid |
| T2D | Type-2 diabetes |
| T2DM | Type-2 diabetes mellitus |
| T300 | Imfinzi plus Imjudo |
| T790M | Threonine 790 substitution with methionine |
| TACE | Transarterial chemoembolization |
| tBRCAm | Tumour (somatic) BRCA-mutated |
| TCE | T-cell engager |
| TCR | T-cell receptor |
| TCR-T | T-cell receptor therapy |
| TDR | Tumour drivers and resistance |
| TEAE | Treatment-emergent adverse event |
| TESAE | Treatment-emergent serious adverse event |
| TFST | Time to first subsequent therapy or death |
| TGFbetaRIIDN | Transforming growth factor-beta RIIDN |
| THP | Paclitaxel, trastuzumab and pertuzumab |
| TID | Three times per day |
| TIGIT | T-cell immunoreceptor with Ig and ITIM domains |
| TIM3 | T-cell immunoglobulin and mucin domain 3 |
| TIM-3 | T-cell immunoglobulin and mucin domain-containing protein |
| TJC | Tender joint count |
| TKI | Tyrosine kinase Inhibitor |
| TLR | Toll-like receptor 9 |
| TMA | Thrombotic microangiopathy |
| Tmax | Time to reach maximum observed plasma concentration |
| TNBC | Triple negative breast cancer |
| TNF | Tumour necrosis factor |
| TNSALP | Tissue-nonspecific alkaline phosphatase |
| TOP1i | Topoisomerase 1 inhibitor |
| TP53 | Tumour protein 53 |
| TP53 R175H | Tumour protein p53 with arginine at position 175 is replaced with histidine |
| TPS | Tumour proportion score |
| Treg | Regulatory T-cell |
| TROP2 | Trophoblast cell surface antigen 2 |
| TSLP | Thymic stromal lymphopoietin |
| TTD | Time to treatment discontinuation |
| TTF | Time to treatment failure |
| TTNT | Time to next therapy |
| TTP | Time to tumour progression |
| TTR | Time to treatment response |
| TTR | Transthyretin |
| u/r HTN | Uncontrolled or treatment resistant hypertension |
| UACR | Urinary albumin/creatinine ratio |
| UK | United Kingdom |
| ULN | Upper limit of normal |
| u-LTE4 | Urinary leukotriene E4 |
| UMEC | Umeclidinium |
| UPCR | Urine protein creatinine ratio |
| URAT1 | Uric acid transporter 1 |
| US | United States |
| V&I | Vaccines and Immune Therapies |
| VEGF | Vascular endothelial growth factor |
| VHH | Single domain antibody |
| VLP | Virus-like particle |
| XELOX | Oxaliplatin and capecitabine |



As of 6 February 2025

Appendix: Glossary.