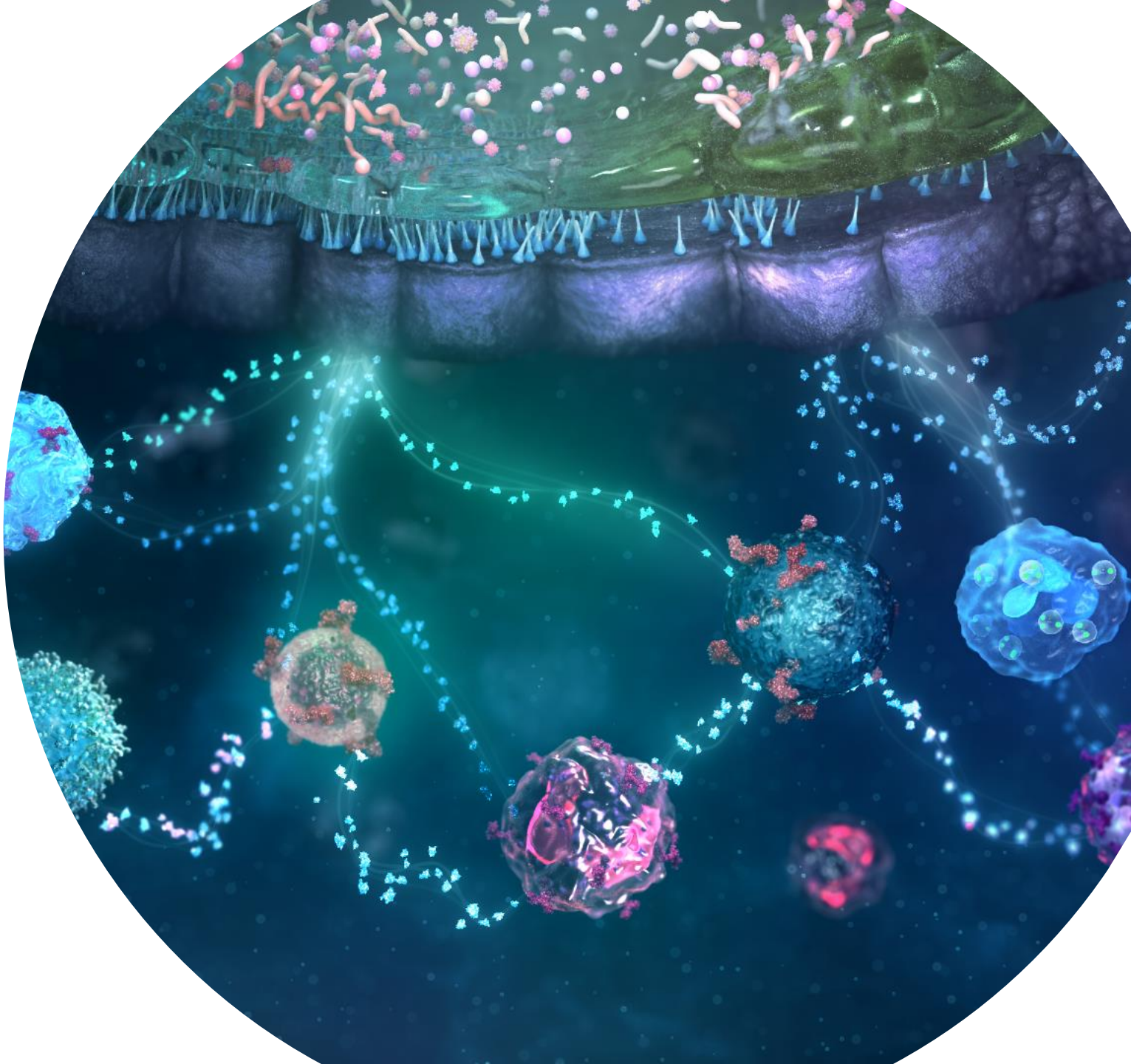
AstraZeneca

Clinical Trials Appendix

H1 2023 Results Update



Key upcoming pipeline catalysts: 2023 and 2024

Oncology BioPharmaceuticals Rare Disease

	H2 2023	H1 2024	H2 2024
Regulatory decision[1,2]	***Lynparza*** – prostate cancer (1L) (PROpel) (JP) ***Enhertu*** – HER2m NSCLC (2L+) (DESTINY-Lung01, DESTINY-Lung02) (EU, JP) **capivasertib** – HR+/HER2-neg breast cancer (2L) (CAPItello-291) ***Enhertu*** – HER2+ breast cancer (3L) (DESTINY-Breast02) (EU) ***Calquence*** – CLL (ASCEND) (CN) ***Forxiga*** – HFpEF (DELIVER) (CN) **eplontersen** – hATTR-PN (NEURO-TTRansform) (US) ***Ultomiris*** – NMOSD (CHAMPION-NMOSD) (US) ***Soliris*** – NMOSD (CN)	***Enhertu*** – HER2+ breast cancer (3L) (DESTINY-Breast02) (US) ***Imfinzi*** – biliary tract cancer (TOPAZ-1) (CN) ***Beyfortus*** – RSV (MELODY-MEDLEY) (JP, CN) **danicopan** – PNH with extravascular haemolysis	***Fasenra*** – asthma (MIRACLE) (CN)
Key Phase III data readouts	***Imfinzi*** – NSCLC (unresectable, Stg. III) (PACIFIC-2) ***Imfinzi*** – SCLC (limited-stage) (ADRIATIC) ***Imfinzi*** – liver cancer (locoregional) (EMERALD-1) **capivasertib** – TNBC (locally adv./met.) (CAPItello-290) **Dato-DXd** – HR+/HER2- breast cancer (inoperable and/or met.) (TROPION-Breast01) ***Fasenra*** – EGPA (MANDARA) **AZD3152** – prevention of COVID-19 (SUPERNOVA)	***Tagrisso*** – EGFRm NSCLC (unresectable Stg. III) (LAURA) ***Imfinzi*** – bladder cancer (1L) (NILE) ***Enhertu*** – high-risk HER2+ early breast cancer (non-met.) (DESTINY-Breast11) ***Enhertu*** – HER2-low breast cancer (2L) (DESTINY-Breast06) ***Fasenra*** – HES (NATRON) ***Ultomiris*** – dermatomyositis (ALXN1210-DM-310) **acoramidis** – ATTR-CM (ALXN2060-TAC-302)	***Tagrisso*** – EGFRm NSCLC (resectable, Stg. II/III) (NeoADAURA) ***Imfinzi*** – GC/GEJC (resectable) (MATTERHORN) ***Imfinzi*** – liver cancer (adjuvant) (EMERALD-2) ***Lynparza*** – PARP BRCAwt ovarian cancer (1L) (MONO-OLA1) ***Orpathys*** – NSCLC with MET exon 14 mutations (locally adv./met.) **Dato-DXd** – TNBC (locally rec. inop./met.) (TROPION-Breast02) ***Enhertu*** – HER2+ breast cancer (1L) (DESTINY-Breast09) ***Breztri*** – moderate asthma (VATHOS) ***Breztri*** – mild to moderate asthma (LITHOS) ***Fasenra*** – CRwNP (ORCHID) ***Tezspire*** – chronic rhinosinusitis with nasal polyps (WAYPOINT) ***Tezspire*** – severe asthma (DIRECTION) ***Koselugo*** – NF1-PN (KOMET) **anselamimab** – AL amyloidosis (Mayo Stg. IIIb) (CAEL101-301)

[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 28 July 2023.
Appendix: Glossary.

Clinical Trials Appendix: selected highlights

	BioPharmaceuticals	Oncology	Rare Disease
Approved medicines: key LCM	Saphnelo® (anifrolumab-fnia) Intravenous Use 300 mg/vial Fasenra® (benralizumab) Subcutaneous Injection 30 mg farxiga® (dapagliflozin) TEZSPIRE™ (tezepelumab-ekko) Subcutaneous Injection 210 mg	TAGRISSO® osimertinib ENHERTU® fam-trastuzumab deruxtecan-nxki 20 mg/mL INJECTION FOR INTRAVENOUS USE CALQUENCE® (acalabrutinib) 100 mg capsules IMFINZI® durvalumab Injection for Intravenous Use 50 mg/mL Lynparza™ olaparib	ULTOMIRIS® (ravulizumab-cwvz)
Next-wave pipeline	tozorakimab (IL-33) AZD3152 (COVID-19 LAAB) eplontersen (LICA) mitiperstat (MPO) baxdrostat (aldosterone synthase inhibitor)	Dato-DXd (TROP2 ADC) capivasertib (AKT) camizestrant (ngSERD) AZD5305 (PARP-1sel) *bispecific mAbs:* volrustomig (PD-1/CTLA-4); rilvegostomig (PD 1/TIGIT); sabestomig (PD-1/TIM3)	vemircopan (oral Factor D) gefurulimab (C5 mini-body) ALXN1850 (ngHPP)

As of 28 July 2023.
Appendix: Glossary.

Project movement since Q1 2023 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
NME **ALXN1920** kidney-targeted factor H fusion protein nephrology **AZD5335** anti-FRα TOP1i ADC ovarian cancer, lung adenocarcinoma **AZD9550#** GLP-1R glucagon dual agonist non-alcoholic steatohepatitis **NT-125** autologous, fully-individualized, multi-specific TCR therapy targeting neoantigens solid tumours	**NME** **AZD2693** NASH resolution non-alcoholic steatohepatitis **AZD3427** relaxin mimetic heart failure **IPH5201 + *Imfinzi*#** CD39 + PD-L1 neo-adjuvant/adjuvant NSCLC **tarperprumig** anti-properdin bispecific sickle cell disease		**NME** **capivasertib + *Faslodex* CAPItello-291#1** AKT inhibitor + *Faxlodex* (2L) and beyond in AI-resistant locally advanced (inoperable) or metastatic breast cancer
Removed from Phase I	**Removed from Phase II**	**Removed from Phase III**	**Removed from registration**
NME **AZD0186#** GLP1R agonism type-2 diabetes **AZD0466#** BCL2/xL haematological malignancies **AZD3366** CD39L3 cardiovascular disease	**NME** **AZD4573** CDK9 inhibitor haematological malignancies **elarekibep#** inhaled IL-4Ra asthma **Additional indication** **AZD4573 + *Calquence*** CDK9 inhibitor + BTK inhibitor haematological malignancies **brazikumab EXPEDITION** IL-23 mAb ulcerative colitis	**NME** **brazikumab INTREPID** IL-23 mAb Crohn's disease **Lifecycle management** ***Fasenra* FJORD** IL-5R mAb bullous pemphigoid **roxadustat#** hypoxia-inducible factor prolyl hydroxylase inhibitor anaemia in myelodysplastic syndrome	**Life-cycle management** ***Ultomiris* CHAMPION-NMOSD2** anti-complement C5 mAb neuromyelitis optica spectrum disorder

Phase progressions based on first patient dose achievement
#Partnered and/or in collaboration [1]Submission accepted [2]Approved
As of 28 July 2023.
Appendix: Glossary.

Q2 2023 Oncology new molecular entity[1] pipeline

Phase I
6 New Molecular Entities

- AZD1390
  ATM glioblastoma
- AZD5335
  anti-FRα TOP1i ADC
  ovarian cancer, lung adenocarcinoma
- AZD9574
  PARP inhibitor advanced solid malignancies
- AZD9592
  EGFR/cMET solid tumours
- NT-125
  autologous, fully-individualised, multi-specific TCR therapy targeting neoantigens solid tumours
- volrustomig + lenvatinib
  PD-1/CTLA-4 + VEGF advanced RCC

Phase II
12 New Molecular Entities

- AZD0171 + *Imfinzi* + CTx
  anti-LIF + PD-L1 + CTx metastatic PDAC (1L)
- AZD5305
  PARP1sel solid tumours
- AZD7789
  PD1/TIM3 bispecific mAb solid tumours, haematological malignancies
- AZD8205
  B7-H4 targeting ADC solid tumours
- camizestrant
  SERD HR+ breast cancer
- capivasertib
  AKT prostate cancer
- ceralasertib
  ATR solid tumours
- *Imfinzi* + monalizumab#
  PD-L1+NKG2A solid tumours
- IPH5201 + *Imfinzi*#
  CD39 + PD-L1 neo-adjuvant/adjuvant NSCLC
- oleclumab + CTx or *Imfinzi* + oleclumab + CTx
  CD73 + CTx or PD-L1 + CD73 + CTx metastatic pancreatic cancer
- rilvegostomig ARTEMIDE-01#
  PD1/TIGIT bispecific mAb solid tumours
- volrustomig
  PD-1/CTLA-4 solid tumours

Phase III
17 New Molecular Entities

- camizestrant + CDK4/6i SERENA-6
  SERD + CDK4/6 HR+ HER2- ESR1m breast cancer (1L)
- camizestrant CAMBRIA-1
  SERD HR+ HER2- extended adjuvant breast cancer
- capivasertib + CTx CAPItello-290
  AKT + chemotherapy mTNBC (1L)
- capivasertib + *Faslodex* + palbociclib CAPItello-292
  AKT + *Faslodex* + CDK4/6 triplet in early relapse/ET resistant locally advanced or mBC (1L)
- ceralasertib + *Imfinzi* MONETTE¶
  ATR inhibitor + PDL-1 melanoma
- datopotamab deruxtecan TROPION-Breast02#
  TROP2 ADC TNBC (1L)
- datopotamab deruxtecan TROPION-Lung01#
  TROP2 ADC NSCLC with or without actionable genomic alterations (2L+)
- datopotamab deruxtecan TROPION-Lung08#
  TROP2 ADC metastatic NSCLC (1L)
- *Imfinzi* +/- oleclumab +/- monalizumab PACIFIC-9#
  PD-L1+NKG2A or PD-L1+CD73 unresectable NSCLC (Stg. III)
- camizestrant + palbociclib SERENA-4
  SERD+CDK4/6 HR+ HER2- breast cancer (1L)
- capivasertib + abiraterone CAPItello-281
  AKT + abiraterone PTEN deficient mHSPC
- capivasertib + docetaxel CAPItello-280
  AKT + docetaxel mCRPC prostate cancer
- ceralasertib + *Imfinzi* LATIFY
  ATR inhibitor + PDL-1 NSCLC
- datopotamab deruxtecan TROPION-Breast01#
  TROP2 ADC HR+ HER2- breast cancer (2-3L)
- datopotamab deruxtecan TROPION-Breast03#
  TROP2 ADC adjuvant residual disease TNBC
- datopotamab deruxtecan TROPION-Lung07#
  TROP 2 ADC NSCLC PD-L1 <50% non-squamous (1L)
- datopotamab deruxtucan AVANZAR#
  TROP 2 ADC NSCLC, squamous and non-squamous NSCLC (1L), TROP2 BM+

Under review
1 New Molecular Entity

- capivasertib + *Faslodex* CAPItello-291
  AKT+fulvestrant 2L and beyond in AI-resistant locally advanced or mBC

Phase progressions based on first patient dose achievement
[1]Includes additional indications for assets where the lead is not yet launched
#Partnered and/or in collaboration ¶Registrational Phase II trial
As of 28 July 2023.
Appendix: Glossary.

Precision medicine approach being explored

Q2 2023 Oncology lifecycle management[1] pipeline

Phase I
2 Projects

- *Enhertu* (platform) DESTINY-Breast08# HER2 ADC HER2-low breast cancer
- *Tagrisso* + (*Koselugo* or *Orpathys*) TATTON# EGFR+MEK/MET advanced EGFRm NSCLC

Phase II
11 Projects

- *Enhertu* (platform) DESTINY-Breast07# HER2 ADC HER2+ breast cancer
- *Enhertu* DESTINY-PanTumour01# HER2 ADC HER2m tumours
- *Enhertu* DESTINY-PanTumour02# HER2 ADC HER2 expressing solid tumours
- *Imfinzi* (platform) BEGONIA PD-L1 1L metastatic TNBC
- *Imfinzi* (platform) COAST# PD-L1+multiple novel therapies NSCLC
- *Imfinzi* (platform) HUDSON PD-L1+multiple novel therapies post IO non-small cell lung cancer
- *Imfinzi* (platform) NeoCOAST# PD-L1+multiple novel therapies NSCLC
- *Imfinzi* + *Lynparza* ORION# PD-L1 + PARP mNSCLC (1L)
- *Lynparza* (basket) LYNK002# PARP HRRm cancer
- *Tagrisso* + *Orpathys* SAVANNAH# EGFR+MET advanced EGFRm NSCLC
- *Tagrisso* ORCHARD platform trial# EGFR + multiple novel therapies EGFRm osimertinib-resistant NSCLC (2L)

Phase III
33 Projects

- *Calquence* + R-CHOP ESCALADE BTK + R-CHOP DLBCL (1L)
- *Enhertu* DESTINY-Breast05# HER2 ADC HER2+ post-neoadjuvant high-risk breast cancer
- *Enhertu* DESTINY-Breast09# HER2 ADC HER2+ breast cancer (1L)
- *Imfinzi* + CRT PACIFIC-5 (China)# PD-L1 + CRT locally-advanced NSCLC (Stg. III)
- *Imfinzi* + CTx neoadjuvant AEGEAN PD-L1 + CTx locally-advanced NSCLC (Stg. II-III)
- *Imfinzi* + FLOT MATTERHORN# PD-L1 + CTx neoadjuvant/adjuvant gastric cancer
- *Imfinzi* + *Imjudo* + TACE +/- lenvatinib EMERALD-3 PD-L1 + CTLA4 + VEGF +/- chemo-embolization locoregional HCC
- *Imfinzi* post-SBRT PACIFIC-4# PD-L1 mAb post-SBRT NSCLC (Stg. I/II)
- *Lynparza* + *Imfinzi* + bevacizumab DUO-O# PARP + PD-L1 + VEGF ovarian cancer (1L)
- *Tagrisso* LAURA EGFR inhibitor EGFRm NSCLC (Stg. III)
- *Tagrisso* + *Orpathys* SAFFRON# EGFR + MET advanced EGFRm NSCLC
- *Calquence* + venetoclax + obinutuzumab AMPLIFY# BTK + BCL-2 + anti-CD20 CLL (1L)
- *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* + CTx NIAGARA PD-L1 + CTx muscle invasive bladder cancer
- *Imfinzi* + EV +/- *Imjudo* VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 MIBC
- *Imfinzi* + VEGF + TACE EMERALD-1# PD-L1 + VEGF + TACE locoregional HCC
- *Imfinzi* +/- *Imjudo* + CRT ADRIATIC# PD-L1 +/- CTLA-4 + CRT LS-SCLC (1L)
- *Lynparza* + *Imfinzi* DUO-E# PARP + PD-L1 endometrial cancer (1L)
- *Orpathys* + *Imfinzi* SAMETA# MET + PD-L1 papillary renal cell carcinoma (1L)
- *Tagrisso* +/- CTx neoadjuvant NeoADAURA EGFR+/-CTx resectable EGFRm NSCLC (Stg. II/III)
- *Calquence* ECHO# BTK inhibitor MCL (1L)
- *Enhertu* DESTINY-Breast06# HER2 ADC post-ET HER2-low/HR+ breast cancer (2L)
- *Enhertu* DESTINY-Lung04# HER2 ADC HER2m NSCLC (1L)
- *Imfinzi* + CRT PACIFIC-2# PD-L1 + CRT locally-advanced NSCLC (Stg. III)
- *Imfinzi* + domvanalimab (AB154) PACIFIC-8# PD-L1 + TIGIT + CTx unresectable NSCLC (Stg. III)
- *Imfinzi* + *Imjudo* + SoC NILE PD-L1 + CTLA-4 + SoC urothelial cancer (1L)
- *Imfinzi* + VEGF EMERALD-2# PD-L1 + VEGF adjuvant HCC
- *Imfinzi* POTOMAC PD-L1 non-muscle invasive bladder cancer
- *Lynparza* MONO-OLA1# PARP BRCAwt ovarian cancer (1L)
- *Tagrisso* + CTx FLAURA2 EGFR + chemo advanced EGFRm NSCLC (1L)
- *Tagrisso* ADAURA2 adjuvant EGFRm NSCLC following complete tumour resection (Stg. Ia/II to Ia/III)

Under review
1 Project

- *Enhertu* DESTINY-Breast02# HER2 ADC HER2+ breast cancer

Phase progressions based on first patient dose achievement
[1]Includes significant lifecycle management projects and parallel indications for assets beyond Phase III
#Partnered and/or in collaboration ¶Registrational Phase I/III trial
As of 28 July 2023.
Appendix: Glossary.

● Precision medicine approach being explored

Q2 2023 BioPharmaceuticals new molecular entity[1] pipeline

Phase I
14 New Molecular Entities

- AZD0780
  PCSK9 dyslipidemia
- AZD2373
  podocyte health nephropathy
- AZD4041#
  orexin 1 receptor antagonist opioid use disorder
- AZD4604
  inhaled JAK1 asthma
- AZD5055
  porcupine inhibitor idiopathic pulmonary fibrosis
- AZD5462#
  RXFP1 agonist heart failure
- AZD6234
  peptide obesity with related comorbidities
- AZD6793
  IRAK4 inhibitor inflammatory diseases
- AZD7503
  ASO non-alcoholic steatohepatitis
- AZD7798
  humanised monoclonal antibody targets T cells subset Crohn's disease
- AZD8630#
  inhaled TSLP FAb asthma
- AZD9550
  GLP-1R glucagon dual agonist non-alcoholic steatohepatitis
- MEDI0618*
  PAR2 antagonist migraine
- MEDI1814#
  amyloid beta mAb Alzheimer's disease

Phase II
13 New Molecular Entities

- atuliflapon
  FLAP asthma
- AZD2693
  NASH resolution non-alcoholic steatohepatitis
- AZD3427
  relaxin mimetic heart failure
- balcinrenone/dapagliflozin
  MR+SGLT2 heart failure with CKD
- baxdrostat
  aldosterone synthase inhibitor hypertension
- MEDI1341#
  alpha synuclein mAb multiple system atrophy/Parkinson's disease
- MEDI6570
  LOX-1 CV disease
- MEDI7352
  NGF/TNF OA pain / PDN
- mitiperstat
  MPO HFpEF / NASH
- mitiperstat
  myeloperoxidase COPD
- tozorakimab
  IL-33 diabetic kidney disease
- tozorakimab FRONTIER 3
  IL-33 asthma
- zibotentan/dapagliflozin
  endothelin A receptor antagonist + SGLT2 CKD / liver cirrhosis

Phase III
4 New Molecular Entities

- AZD3152 SUPERNOVA¶
  SARS-CoV-2 LAAB prevention of COVID-19
- eplontersen#
  LICA ATTR-cardiomyopathy
- tozorakimab OBERON TITANIA PROSPERO
  IL-33 COPD
- tozorakimab TILIA
  IL-33 acute respiratory failure

Under review
1 New Molecular Entity

- eplontersen#
  LICA hATTR-polyneuropathy

Phase progressions based on first patient dose achievement
[1]Includes additional indications for assets where the lead is not yet launched
#Partnered and/or in collaboration *Phase I/IIa ¶Registrational Phase I/III trial
As of 28 July 2023.
Appendix: Glossary.

Precision medicine approach being explored

Q2 2023 BioPharmaceuticals lifecycle management[1] pipeline

Phase I	Phase II	Phase III	Under review
0 Projects	3 Projects	12 Projects	0 Projects
	Andexxa anti-factor Xa reversal urgent surgery	*Breztri*/Trixeo (PT010) KALOS LOGOS LABA/LAMA/ICS asthma	
	roxadustat # HIFPH anaemia chemotherapy induced anaemia	*Farxiga* DAPA-MI SGLT-2 prevention of HF and CV death following a myocardial infarction	
	Tezspire COURSE# TSLP chronic obstructive pulmonary disease	*Fasenra* RESOLUTE# IL-5R chronic obstructive pulmonary disease	
		Fasenra MANDARA IL-5R eosinophilic granulomatosis with polyangiitis	
		Fasenra NATRON IL-5R hypereosinophilic syndrome	
		Fasenra ORCHID# IL-5R nasal polyps	
		Lokelma DIALIZE-Outcomes potassium binder CV outcomes in patients on chronic haemodialysis with hyperkalaemia	
		Lokelma STABILIZE-CKD potassium binder hyperkalaemia in CKD	
		Saphnelo IRIS# Type I IFN receptor mAb lupus nephritis	
		Saphnelo TULIP-SC# Type I IFN receptor SLE SC	
		Tezspire CROSSING# TSLP eosinophilic esophagitis	
		Tezspire WAYPOINT# TSLP nasal polyps	

Phase progressions based on first patient dose achievement

[1]Includes significant lifecycle management projects and parallel indications for assets beyond Phase III

[#]Partnered and/or in collaboration [¶]Registrational Phase I/III trial

As of 28 July 2023.

Appendix: Glossary.

Precision medicine approach being explored

Q2 2023 Rare Disease pipeline[1]

Phase I
6 Projects

- ALXN1850
  next gen TNSALP ERT hypophosphatasia
- ALXN1910
  next gen TNSALP ERT bone metabolism
- ALXN1920
  kidney-targeted factor H fusion protein nephrology
- ALXN2030
  siRNA targeting complement C3 nephrology
- ALXN2080
  oral factor D healthy volunteers
- ALXN2220 (NI006)#
  TTR depleter transthyretin amyloid cardiomyopathy

Phase II
7 Projects

- danicopan
  factor D geographic atrophy
- tarperprumig
  anti-properdin bispecific sickle cell disease
- *Ultomiris*
  anti-complement C5 mAb dermatomyositis
- *Ultomiris*
  anti-complement C5 mAb proliferative lupus nephritis or immunoglobulin A nephropathy
- vemircopan
  oral factor D inhibitor paroxysmal nocturnal haemoglobinuria
- vemircopan
  oral factor D inhibitor generalized myasthenia gravis
- vemircopan
  oral factor D proliferative lupus nephritis or immunoglobulin A nephropathy

Phase III
5 Projects

- acoramidis#
  oral TTR stabilizer transthyretin amyloid cardiomyopathy
- anselamimab
  fibril-reactive mAb AL amyloidosis
- gefurulimab
  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

Under review
1 Project

- danicopan
  factor D PNH with clinically significant extravascular haemolysis

Phase progressions based on first patient dose achievement
[1]Includes new molecular entities and significant lifecycle management projects
#Partnered and/or in collaboration ¶Registrational Phase II/III trial
As of 28 July 2023.
Appendix: Glossary.

● Precision medicine approach being explored

Designations in our pipeline

3 Accelerated approvals

- *Andexxa* acute major bleed (US)
- *Calquence* MCL (1L) (US)
- *Beyfortus* RSV mAb-YTE (EU)

12 Breakthrough / PRIME[1] / Sakigake[2]

- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (CN)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (EU)[1]
- *Calquence* CLL ELEVATE-TN, ASCEND (US)
- *Calquence* MCL (1L) (US)
- danicopan PNH-EVH (US)
- danicopan PNH-EVH (EU)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (US)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (JP)[2]
- *Enhertu* HER2m NSCLC (2L+) DESTINY-Lung01 (US)
- *Koselugo* NF1 SPRINT (US)
- *Tezspire* asthma NAVIGATOR (US)

8 Fast Track

- anselamibab AL amyloidosis (US)
- AZD3427 relaxin mimetic heart failure (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- camizestrant HR+ HER2- ESR1m breast cancer (1L) SERENA-6 (US)
- capivasertib+ *Faslodex* HR+ breast (2L+) CAPItello-291 (US)
- *Lokelma* ESRD DIALIZE-OUTCOMES (US)
- *Saphnelo* SLE (US)
- tozorakimab acute respiratory failure (US)
- *Orpathys* + *Tagrisso* NSCLC SAVANNAH/SAFFRON (US)

14 Priority Review

- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (CN)
- *Calquence* MCL (1L) (US)
- capivasertib+ *Faslodex* HR+ breast (2L+) CAPItello-291 (US)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (US)
- *Enhertu* HER2m NSCLC (2L+) DESTINY-Lung01 (US)
- *Imfinzi* + CTx BTC (1L) (TOPAZ-1) (US)
- *Imfinzi* + *Imjudo* HCC (1L) (HIMALAYA) (US)
- *Koselugo* NF1 SPRINT (US)
- *Koselugo* NF1 SPRINT (CN)
- *Lynparza* + abiraterone all-comers mCRPC (1L) PROpel (US)
- *Lynparza* gBRCAm adj. breast OlympiA (US)
- *Roxadustat* chronic kidney disease (CN)
- *Tezspire* asthma NAVIGATOR (US)
- *Ultomiris* gMG (US)

27 Orphan

- *Andexxa* acute major bleed (JP)
- anselamibab AL amyloidosis (US)
- anselamibab AL amyloidosis (EU)
- *Calquence* CLL (1L) (US)
- *Calquence* CLL (1L) (EU)
- *Calquence* MCL (1L) (US)
- danicopan PNH (US)
- danicopan PNH (EU)
- *Enhertu* HER2+/HER2-low gastric (3L) DESTINY-Gastric01 (US)
- eplontersen transthyretin-mediated amyloidosis (US)
- *Fasenra* EGPA MANDARA (US)
- *Fasenra* HES NATRON (US)
- *Imfinzi* + CTx biliary tract (1L) TOPAZ-1 (US)
- *Imfinzi* + CTx biliary tract (1L) TOPAZ-1 (JP)
- *Imfinzi* +/- *Imjudo* HCC (1L) (EU)
- *Imfinzi* +/- *Imjudo* HCC (1L) (US)
- *Koselugo* NF1 SPRINT (US)
- *Koselugo* NF1 SPRINT (EU)
- *Koselugo* NF1 SPRINT (JP)
- *Lynparza* gBRCAm adj. breast OlympiA (JP)
- tarperprumig sickle cell disease (EU)
- *Tezspire* EoE CROSSING (US)
- *Ultomiris* DM (US)
- *Ultomiris* HSCT-TMA (US)
- *Ultomiris* s.c. PNH (US)
- vemircopan PNH (US)
- vemircopan PNH (EU)

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

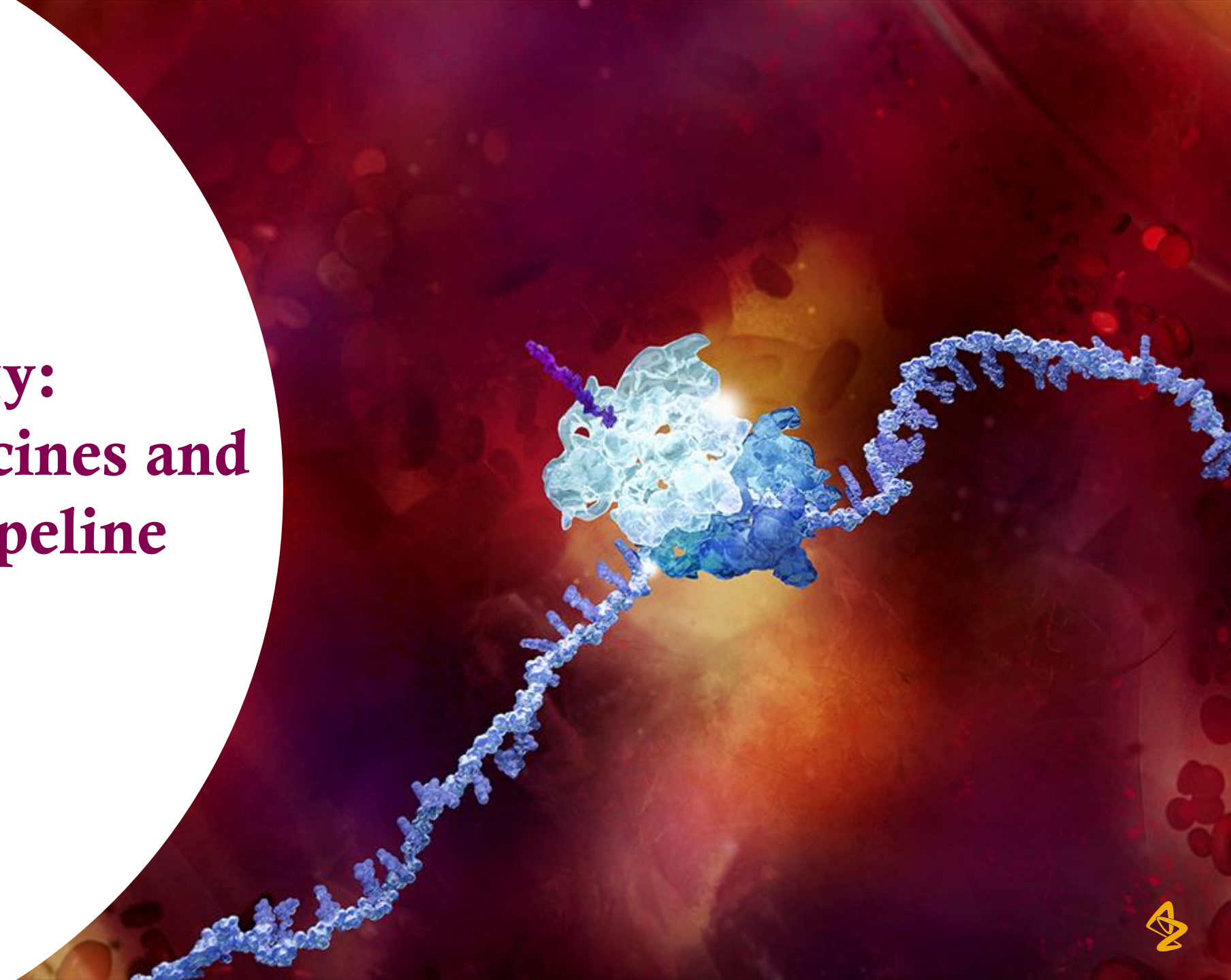
PRIORITY REVIEW DESIGNATION is the US FDA's goal to take action on an application within 6 months

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

Oncology BioPharmaceuticals Rare Disease

NOTE: excludes designations for projects which have launched in all applicable major markets
As of 28 July 2023.
Appendix: Glossary.

Oncology: approved medicines and late-stage pipeline



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III LAURA NCT03521154	Maintenance therapy in patients with locally advanced, unresectable EGFRm Stage III NSCLC whose disease has not progressed following platinum-based chemoradiation therapy	200	• Arm 1: *Tagrisso* • Arm 2: placebo • Global trial – 17 countries	• Primary endpoint: PFS (BICR) • Secondary endpoints: CNS PFS, OS, DoR, ORR and DCR	• FPCD: Q4 2018 • Data anticipated: H1 2024
Phase III ADAURA2 NCT05120349	Adjuvant EGFRm NSCLC Stage IA2 to IA3 following complete tumour resection	380	• Arm 1: *Tagrisso* • Arm 2: placebo	• Primary endpoint: DFS • Secondary endpoints: DFS Rate, OS, OS rate and QoL	• FPCD: Q2 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III NeoADAURA NCT04351555	Neoadjuvant EGFRm NSCLC	351	• Arm 1: placebo + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: *Tagrisso* + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 3: *Tagrisso* • Global trial – 23 countries	• Primary endpoint: mPR • Secondary endpoints: cPR, EFS, DFS and OS	• FPCD: Q1 2021 • Data anticipated: H2 2024
Phase III FLAURA2 NCT04035486	1L EGFRm NSCLC	586	• Arm 1: *Tagrisso* + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: *Tagrisso* • Global trial – 23 countries	• Primary endpoint: PFS • Secondary endpoints: OS, LOS, ORR DoR, depth of response, PFS2, QoL and PK parameters	• FPCD: Q4 2019 • Data readout: Q2 2023 • Primary endpoint met
Phase III COMPEL NCT04765059	EGFRm metastatic NSCLC patients who have progressed extracranially following 1L treatment with Tagrisso	204	• Arm 1: *Tagrisso* + pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: placebo + pemetrexed/carboplatin or pemetrexed/cisplatin • Global trial	• Primary endpoint: PFS • Secondary endpoints: intracranial PFS, extracranial PFS and OS	• FPCD: Q3 2021 • Data anticipated: H2 2024
Phase III SAFFRON NCT05261399 Partnered (HUTCHMED)	EGFR-mutated, 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* • Arm2: pemetrexed with either cisplatin or carboplatin	• Primary enpoint: PFS • Secondary endpoints: OS, ORR, PK, DCR and DoR	• FPCD: Q3 2022 • Data anticipated: >2024
Phase III SANOVO NCT05009836 Partnered (HUTCHMED)	1L EGFRm, MET+ locally advanced or metastatic NSCLC	320	• Arm 1: *Tagrisso* + *Orpathys* • Arm 2: *Tagrisso* + placebo	• Primary endpoint: PFS	• FPCD: Q3 2021 • Data anticipated: H2 2024

As of 28 July 2023.
Appendix: Glossary.

Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SACHI** **NCT05015608** **Partnered (HUTCHMED)**	Locally advanced or metastatic NSCLC with MET amplification after failure of the first-line EGFR inhibitor therapy	250	• Arm 1: *Tagrisso* + *Orpathys* • Arm 2: pemetrexed + platinum	• Primary endpoint: PFS	• FPCD: Q3 2021 • Data anticipated: H2 2024
Phase II **SAVANNAH** **NCT03778229**	EGFRm/MET+, locally advanced or metastatic NSCLC who have progressed following treatment with Tagrisso	360	• Protocol v1-6: single-arm, open-label trial • Protocol v7: randomised, double-blind trial • Arm 1: *Tagrisso* + *Orpathys* • Arm 2: placebo + *Orpathys* • Global trial	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR and OS	• FPCD: Q1 2019 • Data anticipated: H1 2024 • Initial data readout: Q2 2020
Phase II **ORCHARD** **NCT03944772**	Advanced EGFRm NSCLC patients who have progressed on first-line Tagrisso treatment	250	• Modular design platform trial: • Module 1: *Tagrisso* + *Orpathys* (cMET) • Module 2: *Tagrisso* + gefitinib (EGFRm) • Module 3: *Tagrisso* + necitumumab (EGFRm) • Module 4: carboplatin + pemetrexed + *Imfinzi* • Module 5: *Tagrisso* + alectinib (ALK) • Module 6: *Tagrisso* + selpercatinib (RET fusion) • Module 7: *Imfinzi* + etoposide + carboplatin or cisplatin • Module 8: *Tagrisso* + pemetrexed + carboplatin or cisplatin • Module 9: *Tagrisso* + *Koselugo* • Module 10: *Tagrisso* + datopotamab deruxtecan • No intervention: observational cohort • Global trial – 9 countries	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR, OS, safety and tolerability	• FPCD: Q3 2019 • Data anticipated: >2024

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Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Gastrointestinal cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III EMERALD-1 NCT03778957	Locoregional HCC	710	• Arm 1: TACE in combination with *Imfinzi* • Arm 2: TACE in combination with *Imfinzi* + bevacizumab • Arm 3: TACE in combination with placebo	• Primary endpoint: PFS (Arm 2 vs. Arm 3) • Secondary endpoints: PFS (Arm 1 vs. Arm 3) and OS	• FPCD: Q1 2019 • LPCD: Q3 2021 • Data anticipated: H2 2023
Phase III EMERALD-2 NCT03847428	HCC (adjuvant)	908	• Arm 1: *Imfinzi* + bevacizumab • Arm 2: *Imfinzi* + placebo • Arm 3: placebo + placebo	• Primary endpoint: RFS (Arm 1 vs. Arm 3) • Secondary endpoints: RFS (Arm 2 vs. Arm 3), OS and RFS at 24 months	• FPCD: Q2 2019 • LPCD: Q2 2022 • Data anticipated: H2 2024
Phase III KUNLUN NCT04550260	Locally advanced, unresectable ESCC	600	• Arm 1: *Imfinzi* + definitive CRT • Arm 2: placebo + definitive CRT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q4 2020 • LPCD: Q3 2023 • Data anticipated: >2024
Phase III MATTERHORN NCT04592913	Resectable GC/GEJC	900	• Arm 1: *Imfinzi* + FLOT • Arm 2: placebo + FLOT	• Primary endpoint: EFS • Secondary endpoints: OS (Arm 1 vs. Arm 2) and pCR (Arm 1 vs. Arm 2)	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data anticipated: H2 2024
Phase III HIMALAYA NCT03298451	1L HCC	1324	• Arm 1: *Imfinzi* + *Imjudo* • Arm 2: *Imfinzi* • Arm 3: sorafenib	• Primary endpoint: OS • Secondary endpoints: PFS, TTP and ORR	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q4 2021
Phase III TOPAZ-1 NCT03875235	1L BTC	810	• Arm 1: *Imfinzi* + gemcitabine + cisplatin • Arm 2: placebo + gemcitabine + cisplatin • Global trial	• Primary endpoint: OS • Secondary endpoints: PFS, ORR and DoR	• FPCD: Q2 2019 • LPCD: Q4 2020 • Data readout: Q4 2021
Phase III EMERALD-3 NCT05301842	Locoregional HCC	525	• Arm 1: TACE + T300 + D + lenvatanib • Arm 2: TACE + T300 + D • Arm 3: TACE	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q2 2022 • Data anticipated: >2024

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Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III AEGEAN NCT03800134	Neoadjuvant NSCLC patients, Stage II and III resected NSCLC (incl. EGFR/ALK positive)	800	• Arm 1: *Imfinzi* + platinum-based chemotherapy • Arm 2: placebo + platinum-based chemotherapy	• Primary endpoints: pCR snd EFS • Secondary endpoints: mPR and DFS	• FPCD: Q1 2019 • Data readout: Q1 2023
Phase III ADJUVANT BR.31 NCT02273375 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 • Arm 2: placebo • Global trial	• Primary endpoint: DFS • Secondary endpoint: OS	• FPCD: Q1 2015 • LPCD: Q1 2020 • Data anticipated: H1 2024
Phase III PACIFIC-2 NCT03519971	Unresected, locally advanced NSCLC	300	• Arm 1: *Imfinzi* i.v. Q4W + chemotherapy/RT • Arm 2: placebo + chemotherapy/RT • Global trial (ex-US)	• Primary endpoint: PFS • Secondary endpoints: OS and ORR	• FPCD: Q2 2018 • LPCD: Q3 2019 • Data anticipated: H2 2023
Phase III PACIFIC-4 NCT03833154	Imfinzi with SBRT in unresected, Stage I/II NSCLC	630	• Arm 1: *Imfinzi* i.v. Q4W with definitive SBRT • Arm 2: placebo with definitive SBRT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q2 2019 • Data anticipated: >2024
Phase III PACIFIC-5 NCT03706690	Unresected, locally advanced NSCLC	360	• Arm 1: *Imfinzi* i.v. Q4W following chemotherapy/RT • Arm 2: placebo following chemotherapy/RT • Global trial (ex-US with China focus)	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2019 • Data anticipated: H2 2024
Phase III PACIFIC-8 NCT05211895 Partnered (Arcus Biosciences)	Unresected, locally advanced NSCLC	860	• Arm 1: *Imfinzi* + domvanalimab following chemotherapy/RT • Arm 2: *Imfinzi* + placebo following chemotherapy/RT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2022 • Data anticipated: >2024
Phase III POSEIDON NCT03164616	1L NSCLC	1000	• Arm 1: *Imfinzi* + chemotherapy • Arm 2: *Imfinzi* + *Imjudo* + chemotherapy • Arm 3: SoC	• Primary endpoints: OS and PFS	• FPCD: Q2 2017 • LPCD: Q4 2018 • Data readout: Q4 2019 • Primary endpoints met

As of 28 July 2023.
Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III ADRIATIC NCT03703297	Limited-stage SCLC 1L following platinum-based concurrent chemoradiation therapy	600	• Arm 1: *Imfinzi* + *Imjudo* (4 doses) • Arm 2: *Imfinzi* • Arm 3: placebo	• Primary endpoints: PFS and OS	• FPCD: Q4 2018 • Data anticipated: H2 2023
Phase III PACIFIC-9 NCT05221840 Partnered (Innate)	Patients with locally advanced (Stage III), unresectable NSCLC who have not progressed following platinum-based CRT	999	• Arm 1: *Imfinzi* + oleclumab • Arm 2: *Imfinzi* + monalizumab + placebo • Arm 3: *Imfinzi* + placebo	• Primary endoint: PFS • Secondary endpoints: OS, ORR, DoR, PFS2 and TFST	• FPCD: Q2 2022 • Data anticipated: >2024
Phase II HUDSON NCT03334617	NSCLC, patients who progressed on an anti-PD-1/PD-L1-containing therapy	521	• Open-label, biomarker-directed, multi-centre trial • Module 1: *Imfinzi* and *Lynparza* • Module 2: *Imfinzi* and danvatirsen • Module 3: *Imfinzi* and ceralasertib • Module 4: *Imfinzi* and vistusertib • Module 5: *Imfinzi* and oleclumab • Module 6: *Imfinzi* and *Enhertu* • Module 7: *Imfinzi* and cediranib • Module 8: ceralasertib • Module 9: *Imfinzi* and ceralasertib • Module 10: *Imfinzi* and ceralasertib • Module 11: ceralasertib	• Primary endpoint: ORR • Secondary endpoints: efficacy including OS, PFS, DCR, safety and tolerability and DoR	• FPCD: Q1 2018 • Data anticipated: >2024
Phase II NeoCOAST NCT03794544	Resectable, early-stage NSCLC	84	• Arm 1: *Imfinzi* • Arm 2: *Imfinzi* + oleclumab • Arm 3: *Imfinzi* + monalizumab • Arm 4: *Imfinzi* + danvatirsen	• Primary endpoint: major pathological response rate	• FPCD: Q1 2019 • LPCD: Q1 2021 • Data readout: Q1 2022

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Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II NeoCOAST-2 NCT05061550	Early stage, resectable NSCLC (Stage II to Stage IIIA)	350	• Open-label trial • Arm 1: *Imfinzi* + oleclumab + platinum doublet chemotherapy • Arm 2: *Imfinzi* + monalizumab + platinum doublet chemotherapy • Arm 3: volrustomig + platinum doublet chemotherapy • Arm 4: datopotamab deruxtecan + single agent platinum chemotherapy • Arm 5: AZD0171 + platinum doublet chemotherapy	• Primary endpoints: pCR, safety	• FPCD: Q2 2022 • Data anticipated: >2024
Phase I/II SCope-D1 NCT04870112	NSCLC, SCLC	18	• Open-label, multi-centre trial • s.c. *Imfinzi*	• Primary endpoints: PK parameters and safety	• FPCD: Q4 2021 • Data anticipated: H2 2023

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Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Other cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III POTOMAC NCT03528694	Non-muscle invasive bladder cancer	1018	• Arm 1: BCG (induction + maintenance) • Arm 2: *Imfinzi* + BCG (induction only) • Arm 3: *Imfinzi* + BCG (induction + maintenance)	• Primary endpoint: DFS	• FPCD: Q2 2018 • LPCD: Q4 2020 • Data anticipated: >2024
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	1063	• Arm 1: *Imfinzi* in combination with gemcitabine + cisplatin, *Imfinzi* maintenance • Arm 2: gemcitabine + cisplatin	• Co-primary endpoints: pCR and EFS	• FPCD: Q4 2018 • LPCD: Q3 2021 • Data anticipated: >2024
Phase III SAMETA NCT05043090	MET-driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	• Arm 1: *Orpathys* + *Imfinzi* • Arm 2: sunitinib • Arm 3: *Imfinzi* monotherapy	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR and DCR	• FPCD: Q4 2021 • Data anticipated: >2024
Phase III NILE NCT03682068	1L bladder cancer	1292	• Arm 1: *Imfinzi* + *Imjudo* + SoC • Arm 2: *Imfinzi* + SoC • Arm 3: SoC	• Primary endpoint: OS	• FPCD: Q4 2018 • LPCD: Q2 2021 • Data anticipated: H1 2024
Phase III VOLGA NCT04960709	Muscle-invasive bladder cancer ineligible to cisplatin	830	• Arm 1: *Imfinzi* + *Imjudo* + enfortumab vedotin • Arm 2: *Imfinzi* + enfortumab vedotin • Arm 3: SoC cystectomy	• Primary endpoints: safety, EFS and pCR • Secondary endpoint: OS	• FPCD: Q4 2021 • Data anticipated: >2024
Phase II BEGONIA NCT03742102	1L mTNBC	240	• Arm 1: *Imfinzi* + paclitaxel • Arm 2: *Imfinzi* + paclitaxel + capivasertib • Arm 5: *Imfinzi* + paclitaxel + oleclumab • Arm 6: *Imfinzi* + *Enhertu* • Arm 7: *Imfinzi* + datopotamab deruxtecan • Arm 8: *Imfinzi* + datopotamab deruxtecan (PDL1-high) • Global trial	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, PFS, DoR, OS, PK and ADA	• FPCD: Q1 2019 • Data anticipated: H2 2024

As of 28 July 2023.
Appendix: Glossary.

Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DUO-O** **NCT03737643**	1L advanced ovarian cancer	1256	• Non-tBRCAm (tumour BRCA) patients • Arm 1: chemotherapy + bevacizumab + *Imfinzi* placebo followed by bevacizumab + *Imfinzi* placebo + *Lynparza* placebo • Arm 2: chemotherapy + bevacizumab + *Imfinzi* followed by bevacizumab + *Imfinzi* + *Lynparza* placebo • Arm 3: chemotherapy + bevacizumab + *Imfinzi* followed by bevacizumab + *Imfinzi* + *Lynparza* • tBRCAm patients • chemotherapy + bevacizumab (optional) + *Imfinzi* followed by bevacizumab (optional) + *Imfinzi* + *Lynparza* • Global trial	• Primary endpoint: PFS • Secondary endpoints: OS and PFS2	• FPCD: Q1 2019 • Data readout: Q2 2023 • Primary endpoint met
Phase III **DUO-E** **NCT04269200**	1L advanced and recurrent endometrial cancer	699	• Arm 1: chemotherapy + *Imfinzi* placebo followed by *Imfinzi* placebo + *Lynparza* placebo • Arm 2: chemotherapy + *Imfinzi* followed by *Imfinzi* + *Lynparza* placebo • Arm 3: chemotherapy + *Imfinzi* followed by *Imfinzi* + *Lynparza* • Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, PFS2, ORR and DoR	• FPCD: Q2 2020 • Data readout: Q2 2023 • Primary endpoint met

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Appendix: Glossary.

Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered (BIG & NRG Oncology)	gBRCAm adjuvant breast cancer	1836	• Arm 1: *Lynparza* BID 12-month duration • Arm 2: placebo 12-month duration • Global trial in partnership with Breast International Group and National Cancer Institute/NRG Oncology	• Primary endpoint: iDFS • Secondary endpoints: distant disease-free survival and OS	• FPCD: Q2 2014 • LPCD: Q2 2019 • Data readout: Q1 2021 • Primary endpoint met
Phase III MONO-OLA1 NCT04884360	BRCAwt advanced ovarian cancer, 1L maintenance	420	• Arm 1: *Lynparza* BID 24-month duration • Arm 2: placebo BID 24-month duration • Global trial – 12 countries	• Primary endpoints: PFS (BRCAwt HRD+ve) and PFS (BRCAwt) • Secondary endpoints: OS, TFST and PFS2	• FPCD: Q3 2021 • Data anticipated: H2 2024

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Appendix: Glossary.

Lynparza (PARP inhibitor)

Other combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **PROpel** **NCT03732820**	1L metastatic castration-resistant prostate cancer	904	• Arm 1: *Lynparza* + abiraterone • Arm 2: placebo + abiraterone • Global trial (including China)	• Primary endpoint: rPFS • Secondary endpoints: OS	• FPCD: Q4 2018 • LPCD: Q3 2022 • Data readout: Q3 2021 • Primary endpoint met
Phase II/III **COCOS (GY005)** **NCT02502266** **Partnered (National Cancer Institute)**	Recurrent platinum R/R ovarian cancer	562	• Arm 1: chemotherapy • Arm 2: cediranib + *Lynparza* • Arm 3: cediranib • Arm 4: *Lynparza* • US and Canada	• Primary endpoints: PFS and OS • Secondary endpoints: ORR, QoL and safety	• FPCD: Q2 2016 • LPCD: Q1 2022 • Data readout: Q3 2023 • Primary endpoint not met
Phase II **LYNK-002** **NCT03742895** **Partnered (Merck Sharp & Dohme LLC)**	HRRm or HRD-positive advanced cancer	390	• Arm 1: *Lynparza* • Global trial	• Primary endpoint: ORR • Secondary endpoints: DOR, OS, PFS, AE and Prog by CA-125	• FPCD: Q1 2019

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Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III DESTINY-Breast02 NCT03523585 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 • Arm 1: *Enhertu* • Arm 2: physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine	• Primacy endpoint: PFS • Secondary endpoints: OS, ORR, DoR and CBR	• FPCD: Q3 2018 • LPCD: Q4 2020 • Data readout: Q3 2022 • Primary endpoint met
Phase III DESTINY-Breast03 NCT03529110 Partnered (Daiichi Sankyo)	HER2-positive, unresectable and/or metastatic breast cancer previously treated with trastuzumab and taxane	524	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: ado-trastuzumab emtansine	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR and CBR	• FPCD: Q3 2018 • LPCD: Q2 2020 • Data readout: Q3 2021 • Primary endpoint met
Phase III DESTINY-Breast04 NCT03734029 Partnered (Daiichi Sankyo)	HER2-low, unresectable and/or metastatic breast cancer	557	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: physician's choice of SoC chemotherapy (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q4 2018 • LPCD: Q4 2020 • Data readout: Q1 2022 • Primary endpoint met
Phase III DESTINY-Breast05 NCT04622319 Partnered (Daiichi Sankyo)	High-risk HER2-positive with residual invasive breast cancer following neoadjuvant therapy	1600	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: ado-trastuzumab emtansine	• Primary endpoint: IDFS • Secondary endpoints: DFS, OS, DRFI and BMFI	• FPCD: Q4 2020 • Data anticipated: >2024
Phase III DESTINY-Breast06 NCT04494425 Partnered (Daiichi Sankyo)	HER2-low, HR+ breast cancer with disease progression on endocrine therapy in the metastatic setting	850	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: investigator's choice SoC chemotherapy (capecitabine, paclitaxel, nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q3 2020 • Data anticipated: H1 2024
Phase III DESTINY-Breast09 NCT04784715 Partnered (Daiichi Sankyo)	HER2-positive, metastatic breast cancer with no prior therapy for advanced or metastatic disease	1134	• Randomised, parallel assignment • Arm 1: *Enhertu* + placebo • Arm 2: *Enhertu* + pertuzumab • Arm 3: SoC	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q2 2021 • Data anticipated: H2 2024

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Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Breast11** **NCT05113251** **Partnered (Daiichi Sankyo)**	High-risk HER2-positive early non-metastatic breast cancer	624	• Randomised, open-label, parallel assignment • Arm 1: *Enhertu* • Arm 2: *Enhertu* followed by THP • Arm 3: doxorubicin and cyclophosphamide followed by THP	• Primary endpoint: pCR • Secondary endpoints: EFS, IDFS and OS	• FPCD: Q4 2021 • Data anticipated: H1 2024
Phase Ib/II **DESTINY-Breast07** **NCT04538742** **Partnered (Daiichi Sankyo)**	HER2-positive metastatic breast cancer	450	• Randomised, open-label, sequential assignment • Arm 1: *Enhertu* • Arm 2: *Enhertu* + *Imfinzi* • Arm 3: *Enhertu* + pertuzumab • Arm 4: *Enhertu* + paclitaxel • Arm 5: *Enhertu* + *Imfinzi* + paclitaxel • Arm 6: *Enhertu* + tucatinib	• Primary endpoints: AE and SAE • Secondary endpoints: ORR, PFS, DoR and OS	• FPCD: Q1 2021 • Data anticipated: >2024
Phase Ib **DESTINY-Breast08** **NCT04556773** **Partnered (Daiichi Sankyo)**	HER2-low metastatic breast cancer	139	• Non-randomised, open-label parallel assignment • Arm 1: *Enhertu* + capecitabine • Arm 2: *Enhertu* + *Imfinzi* + paclitaxel • Arm 3: *Enhertu* + capivasertib • Arm 4: *Enhertu* + anastrozole • Arm 5: *Enhertu* + *Faslodex*	• Primary endpoints: AE and SAE • Secondary endpoints: ORR, PFS, DoR and OS	• FPCD: Q1 2021 • Data anticipated: H2 2023

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Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Gastric cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Gastric04** **NCT04704934** **Partnered (Daiichi Sankyo)**	HER2-positive gastric cancer or gastro-esophageal junction 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 • Arm 1: *Enhertu* • Arm 2: SoC chemotherapy	• Primary endpoint: OS • Secondary endpoints: ORR, DoR, PFS, DcR and safety	• FPCD: Q2 2021 • Data anticipated: >2024
Phase II **DESTINY-Gastric01** **NCT03329690** **Partnered (Daiichi Sankyo)**	HER2-overexpressing advanced gastric or gastroeosophogeal junction adenocarcinoma patients who have progressed on two prior treatment regimens	233	• Randomised, open-label parallel assignment • Arm 1: *Enhertu* • Arm 2: SoC chemotherapy • Two additional open-label patient cohorts with lower levels of HER2 expression • Japan and Korea	• Primary endpoint: ORR • Secondary endpoints: PFS, OS, DoR, DCR, TTF and range of PK parameters	• FPCD: Q4 2017 • LPCD: Q2 2019 • Data readout: Q1 2020 • Primary endpoint met
Phase II **DESTINY-Gastric02** **NCT04014075** **Partnered (Daiichi Sankyo)**	HER2-positive gastric cancer that cannot be surgically removed or has spread, in patients who have progressed on or after trastuzumab containing regimen	79	• Open-label, single group assignment • *Enhertu* • Western population	• Primary endpoint: ORR • Secondary endpoints: PFS, ORR, OS and DoR	• FPCD: Q4 2019 • LPCD: Q4 2020 • Data readout: Q2 2021 • Primary endpoint met
Phase II **DESTINY-Gastric06** **NCT04989816** **Partnered (Daiichi Sankyo)**	HER2-positive gastric cancer or gastro-esophageal junction adenocarcinoma patients who have progressed on two prior treatment regimens	100	• Open-label, single group assignment • *Enhertu* • China only	• Primary endpoint: ORR • Secondary endpoints: PFS, ORR, DCR, OS, DoR and safety	• FPCD: Q2 2021 • Data anticipated: H2 2023
Phase Ib/II **DESTINY-Gastric03** **NCT04379596** **Partnered (Daiichi Sankyo)**	HER2-overexpressing gastric or gastroeosophogeal junction cancer	255	• Open-label, parallel assignment • Part 1: to determine recommended Phase II combination dose • 5 Arms combining *Enhertu* with SoC chemotherapies (5-FU, capecitabine, oxaliplatin) and/or durvalumab • Part 2: to assess efficacy of the selected combinations • Arm 2A: standard chemotherapy • Arm 2B: *Enhertu* monotherapy • Arm 2C: *Enhertu* with chemotherapy • Arm 2D: *Enhertu* with chemotherapy and pembrolizumab • Arm 2E: *Enhertu* and pembrolizumab	• Primary endpoint (Part 1): safety • Primary endpoint (Part 2): ORR • Secondary endpoints: DoR, DCR, PFS, OS, PK parameters and preence of ADAs	• FPCD: Q2 2020 • Data anticipated: H2 2023

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Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DESTINY-Lung04** **NCT05048797** **Partnered (Daiichi Sankyo)**	HER2-mutated, unresectable, locally advanced/metastatic NSCLC	264	• Randomised, parallel group assignment • Arm 1: *Enhertu* • Arm 2: SoC (platinum, pemetrexed and pembrolizumab)	• Primary endpoint: PFS • Secondary endpoints: OS, CNS-PFS, PFS (INV), ORR, DoR, safety, PK parameters, ADA, PRO-tolerability and PRO- pulmonary symptoms	• FPCD: Q4 2021 • Data anticipated: >2024
Phase II **DESTINY-Lung02** **NCT04644237** **Partnered (Daiichi Sankyo)**	HER2-mutated, unresectable and/or metastatic NSCLC	152	• Randomised, parallel group assignment • Arm 1: *Enhertu* 6.4mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS and PK parameters	• FPCD: Q1 2021 • Data readout: Q1 2023 • Primary endpoint met
Phase II **DESTINY-PanTumor02** **NCT04482309** **Partnered (Daiichi Sankyo)**	HER2-expressing tumours	268	• Non-randomised, single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS and OS	• FPCD: Q4 2020 • Data readout: Q1 2023
Phase II **DESTINY-PanTumor01** **NCT04639219** **Partnered (Daiichi Sankyo)**	HER2-mutated tumours	102	• Non-randomised, single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS and PK parameters	• FPCD: Q1 2021 • Data readout: Q2 2023
Phase II **DESTINY-CRC02** **NCT04744831** **Partnered (Daiichi Sankyo)**	HER2-overexpressing advanced or metastatic colorectal cancer	120	• Randomised, parallel group assignment • Arm 1: *Enhertu* 6.4mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoints: ORR, PFS, OS, DoR, DCR and PK parameters	• FPCD: Q1 2021 • Data readout: Q1 2023 • Primary endpoint met

As of 28 July 2023.
Appendix: Glossary.

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib **DESTINY-Lung03** **NCT04686305** **Partnered (Daiichi Sankyo)**	HER2-over-expressing, unresectable and/or metastatic NSCLC	136	• Non-randomised, parallel group assignment • Part 1: to determine recommended combination dose • 3 Arms combine *Enhertu* with SoC chemotherapies (cisplatin, carboplatin or pemetrexed) and *Imfinzi*; Arm 1D: *Enhertu* monotherapy arm • Part 2: to assess efficacy of the selected combinations • Arm 1: *Enhertu* + cisplatin + *Imfinzi* • Arm 2: *Enhertu* + carboplatin + *Imfinzi* • Arm 3: *Enhertu* + pemetrexed + *Imfinzi* • Arm 4: *Enhertu* + *Imfinzi*	• Primary endpoint: safety • Secondary endpoints: ORR, DoR, DCR, PFS, OS and PK parameters	• FPCD: Q4 2021 • Data anticipated: H1 2024
Phase Ib **U106** **NCT04042701** **Partnered (Daiichi Sankyo)**	HER2-expressing locally advanced/metastatic breast or NSCLC	115	• Non-randomised, parallel group assignment • *Enhertu* + pembrolizumab • Global trial – 2 countries	• Primary endpoints: DLT and ORR • Secondary endpoints: DoR, DCR, PFS, TTR and OS	• FPCD: Q2 2020 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **AMPLIFY (ACE-CL-311)** **NCT03836261**	Previously untreated CLL	981	• Arm 1: *Calquence* + venetoclax • Arm 2: *Calquence* + venetoclax + obinutuzumab • Arm 3: FCR or BR	• Primary endpoint: IRC PFS (Arm 1 vs. Arm 3) • 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 • Data anticipated: >2024
Phase III **ASCEND (ACE-CL-309)** **NCT02970318**	R/R CLL	306	• Arm 1: *Calquence* • Arm 2: rituximab + idelalisib or bendamustine (investigator's choice)	• Primary endpoint: IRC assessed PFS (Arm 1 vs. Arm 2) • Secondary endpoints: INV-assessed ORR, OS, DoR and PROs	• FPCD: Q4 2017 • Data readout: Q2 2019 • Primary endpoint met
Phase III **ECHO (ACE-LY-308)** **NCT02972840**	Previously untreated MCL	634	• Arm 1: *Calquence* + bendamustine + rituximab • Arm 2: bendamustine + rituximab	• Primary endpoint: PFS by Lugano Classification for NHL • Secondary endpoints: IA, PFS, ORR, DoR, time to response and OS	• FPCD: Q2 2017 • Data anticipated: >2024
Phase III **ESCALADE** **NCT04529772**	DLBCL	600	• *Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine and prednisone	• Primary endpoint: PFS	• FPCD: Q1 2021 • Data anticipated: >2024
Phase III **NCT04075292**	Untreated CLL	155	• Arm 1: *Calquence* • Arm 2: chlorambucil + rituximab	• Primary endpoint: PFS • Secondary endpoints: ORR and DoR	• FPCD: Q1 2020 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib **ACE-LY-106** **NCT02717624**	MCL	61	• *Calquence* in combination with bendamustine and rituxumab • Arm 1: treatment naive • Arm 2: R/R • Arm 3: treatment naïve: *Calquence* + venetoclax + rituxumab	• Primary endpoint: safety	• FPCD: Q2 2016 • LPCD: Q2 2022 • Data readout: Q1 2023
Phase I **ACE-LY-003** **NCT02180711**	R/R follicular lymphoma	89	• Arm 1: *Calquence* • Arm 2: *Calquence* + rituximab • Arm 3: *Calquence* + rituximab + lenolidomide	• Primary endpoint: safety	• FPCD: Q1 2015 • Data anticipated: H1 2024
Phase I **ACE-CL-003** **NCT02296918**	CLL/SLL/PLL	114	• *Calquence* + obinutuzumab • Arm 1: R/R • Arm 2: treatment naïve • *Calquence* + venetoclax + rituxumab • Arm 3: R/R • Arm 4: treatment naïve	• Primary endpoints: safety and ORR • Secondary endpoints: PD, PFS, TTNT and OS	• FPCD: Q4 2014 • Data readout: Q1 2022

As of 28 July 2023.
Appendix: Glossary.

Orpathys (savolitinib, MET inhibitor)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NCT04923945** **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 • *Orpathys*	• Primary endpoint: ORR	• FPCD: Q3 2021 • Data anticipated: H2 2024
Phase II **NCT04923932** **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 • *Orpathys*	• Primary endpoint: ORR • Secondary endpoints: PFS and safety	• FPCD: Q3 2021 • Data anticipated: H2 2024

As of 28 July 2023.
Appendix: Glossary.

capivasertib (AKT inhibitor)

Breast cancer and prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPItello-290 NCT03997123	Locally advanced or metastatic TNBC	924	• Double-blind, randomised, comparative trial • Arm 1: capivasertib + paclitaxel • Arm 2: placebo + paclitaxel	• Primary endpoint: OS	• FPCD: Q3 2019 • Data anticipated: H2 2023
Phase III CAPItello-291 NCT04305496	2L+ AI-resistant locally advanced (inoperable) or metastatic HR+/HER2- breast cancer	834	• Double-blind, randomised, comparative trial • Arm 1: capivasertib + *Faslodex* • Arm 2: placebo + *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2020 • Data readout: Q4 2022 • Both primary endpoints met
Phase III CAPItello-281 NCT04493853	De novo PTEN deficient metastatic hormone sensitive prostate cancer	1000	• Double-blind, randomised, comparative trial • Arm 1: capivasertib + abiraterone • Arm 2: placebo + abiraterone	• Primary endpoint: rPFS	• FPCD: Q3 2020 • Data anticipated: >2024
Phase III CAPItello-292 NCT04862663	1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+/HER2- breast cancer	700	• Double-blind, randomised, comparative trial • Arm 1: capivasertib + palbociclib + *Faslodex* • Arm 2: placebo + palbociclib + *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2021 • Data anticipated: >2024
Phase III CAPItello-280 NCT05348577	mCRPC prostate cancer	790	• Double-blind, randomised, comparative trial • Arm 1: capivasertib + docetaxel • Arm 2: placebo + docetaxel	• Primary endpoint: OS	• FPCD: Q2 2022 • Data anticipated: >2024
Phase II CAPITAL NCT05008055	R/R FL, R/R MZL, R/R MCL	272	• Open-label, non-randomised	• Primary endpoints: ORR and safety • Secondary endpoint: DOR, PFS, OS, safety and PK/PD parameters	• FPCD: Q4 2021 • Trial discontinued due to strategic portfolio prioritisation

As of 28 July 2023.
Appendix: Glossary.

camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2- advanced breast cancer	1342	• Randomised, double-blind, comparative trial • Arm 1: camizestrant + palbociclib • Arm 2: anastrazole + palbociclib	• Primary endpoint: PFS • Secondary endpoints: OS and PFS2	• FPCD: Q1 2021 • Data anticipated: >2024
Phase III SERENA-6 NCT04964934	HR+ HER2- advanced breast cancer	300	• Randomised, double-blind, comparator trial • Arm 1: camizestrant + palbociclib or abemaciclib • Arm 2: anastrazole or letrozole + palbociclib or abemaciclib	• Primary endpoint: PFS • Secondary endpoint: OS and PFS2	• FPCD: Q3 2021 • Data anticipated: >2024
Phase III CAMBRIA-1 NCT05774951	ER+/HER2- 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 • Arm 2: camizestrant • Global trial – 39 countries	• Primary endpoint: IBCFS • Secondary endpoints: IDFS, DRFS and OS	• FPCD: Q1 2023 • Data anticipated: >2024
Phase II SERENA-2 NCT04214288	HR+ advanced breast cancer	240	• Randomised, open-label, parallel-group, multi-centre trial • Arm 1: camizestrant (75mg) • Arm 2: camizestrant (150mg) • Arm 3: camizestrant (300mg) • Arm 4: *Faslodex*	• Primary endpoint: PFS	• FPCD: Q2 2020 • LPCD: Q3 2021 • Data readout: Q4 2022 • Primary endpoint met at 75mg and 150mg doses
Phase II SERENA-3 NCT04588298	HR+ HER2- early breast cancer	132	• Randomised, open-label, parallel-group, multi-centre trial • camizestrant	• Primary endpoint: change in ER expression between pre- and on-treatment tumour biopsies	• FPCD: Q4 2020 • LPCD: Q2 2023 • Data anticipated: H2 2023
Phase I NCT04541433	HR+ HER2- advanced breast cancer	18	• Open-label trial • camizestrant • Japan only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2020 • LPCD: Q1 2022 • Data readout: Q1 2023

As of 28 July 2023.
Appendix: Glossary.

camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I SERENA-1 NCT03616587	HR+ HER2- advanced breast cancer	403	• Escalation phase: open-label multi-centre trial • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), capivasertib, ribociclib (+/- anastrozole) or anastrozole • Expansion phase: randomised expansion cohort(s) • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib, everolimus, abemeciclib (+/- anastrozole), capivasertib, ribociclib (+/- anastrozole) or anastrozole	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and anti-tumour activity	• FPCD: Q4 2018 • Data anticipated: H2 2024
Phase I NCT04818632	HR+ HER2- metastatic breast cancer in Chinese patients	30	• Dose escalation: camizestrant • Dose expansion: • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib • Cohort 3: camizestrant + everolimus • China only	• Primary endpoints: safety and tolerability, PK parameters • Secondary endpoint: anti-tumour activity	• FPCD: Q1 2021 • LPCD: Q1 2023 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Lung01 NCT04656652 Partnered (Daiichi Sankyo)	Previously treated advanced or metastatic NSCLC with or without actionable genomic alterations	590	• Randomised, open-label, parallel assignment • Arm 1: datopotamab deruxtecan • Arm 2: docetaxel • Global trial	• Primary endpoints: PFS and OS • Secondary endpoints: ORR, DoR, TTR, DCR, PK parameters and ADA	• FPCD: Q1 2021 • LPCD: Q4 2022 • Data readout: Q3 2023 • Dual primary endpoint met (PFS)
Phase III TROPION-Lung08 NCT05215340 Partnered (Daiichi Sankyo)	Treatment-naïve patients with PD-L1-high advanced or metastatic NSCLC without actionable genomic alterations	740	• Randomised, open-label • Arm 1: datopotamab deruxtecan + pembrolizumab • Arm 2: pembrolizumab • Global trial	• Primary endpoints: PFS and OS	• FPCD: Q1 2022 • Data anticipated: >2024
Phase III TROPION-Lung07 NCT05555732 Partnered (Daiichi Sankyo)	1L patients with PD-L1 TPS <50% and advanced or metastatic NSCLC without actionable genomic alternations	975	• Randomised, open-label • Arm 1: datopotamab deruxtecan + pembrolizumab + platinum chemotherapy • Arm 2: datopotamab deruxtecan + pembrolizumab • Arm 3: pembrolizumab + pemetrexed + platinum chemotherapy • Global trial	• Primary endpoints: PFS and OS	• FPCD: Q1 2023 • Data anticipated: >2024
Phase III AVANZAR NCT05687266	1L NSCLC	1000	• Arm 1: carboplatin + datopotamab deruxtecan + *Imfinzi* • Arm 2: pembrolizumab • Global trial	• Co-primary endpoints: OS and PFS in TROP2 biomarker-positive	• FPCD: Q1 2023 • Data anticipated: >2024

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Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase II **TROPION-Lung05** **NCT04484142** **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 • datopotamab deruxtecan • Global trial	• Primary endpoint: ORR • Secondary endpoints: DOR, PFS, OS, safety, PK parameters and ADA	• FPCD: Q1 2021 • LPCD: Q1 2022 • Data anticipated: H2 2024
Phase I **TROPION-Lung02** **NCT04526691** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC	145	• Open-label, two-part (dose escalation and dose expansion), sequential assignment • datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy • Global trial – US, Japan, Italy, Spain and Taiwan	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q4 2020 • LPCD: Q2 2023 • Data anticipated: H1 2024
Phase I **TROPION-Lung04** **NCT04612751** **Partnered (Daiichi Sankyo)**	Advanced or metastatic NSCLC	232	• Open-label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + *Imfinzi* +/- platinum chemotherapy • Cohort 1 & 2: datopotamab deruxtecan + *Imfinzi* • Cohort 3 & 4: datopotamab deruxtecan + *Imfinzi* + carboplatin • Cohort 5 & 6: datopotamab deruxtecan + rilvegostomig • Cohort 7 & 8: datopotamab deruxtecan + rilvegostomig + carboplatin • Cohort 9 &10: datopotamab deruxtecan + volrustomig + carboplatin • Cohort 11: datopotamab deruxtecan + volrustomig • Global trial – US, Japan, Taiwan and Belgium	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2021 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Breast01 NCT05104866 Partnered (Daiichi Sankyo)	Inoperable or metastatic HR+ HER2- breast cancer	733	• Open-label, randomised • Arm 1: datopotamab deruxtecan • Arm 2: investigator's choice SoC chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine)	• Primary endpoints: PFS (BICR) and OS • Secondary endpoints: ORR, DoR, PFS (Inv), DCR, PK parameters and ADA	• FPCD: Q4 2021 • LPCD: Q4 2022 • Data anticipated: H2 2023
Phase III TROPION-Breast02 NCT05374512 Partnered (Daiichi Sankyo)	Locally recurrent inoperable or metastatic TNBC	600	• Open-label, randomised • Arm 1: datopotamab deruxtecan • Arm 2: investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate) • Global trial	• Primary endpoints: PFS (BICR) and OS • Secondary endpoints: PFS (Inv), ORR, DoR, PK parameters and ADA	• FPCD: Q2 2022 • Data anticipated: H2 2024
Phase III TROPION-Breast03 NCT05629585 Partnered (Daiichi Sankyo)	Stage I-III TNBC without pathological complete response following neoadjuvant therapy	1075	• Open-label, randomised • Arm 1: datopotamab deruxtecan + *Imfinzi* • Arm 2: datopotamab deruxtecan • Arm 3: investigator's choice of therapy (capecitabine, pembrolizumab, or capecitabine + pembrolizumab) • Global trial	• Primary endpoint: iDFS • Secondary endpoints: DDFS, OS, PK and ADA	• FPCD: Q4 2022 • Data anticipated: >2024

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Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **TROPION-PanTumor03** **NCT05489211** **Partnered (Daiichi Sankyo)**	Endometrial cancer, gastric cancer, mCRPC, ovarian cancer, CRC, bladder and BTC	531	• Sub-study 1 (endometrial cancer); • Sub-study 1a: datopotamab deruxtecan monotherapy • Sub-study 1b: datopotamab deruxtecan + *Imfinzi* • Sub-study 1c: datopotamab deruxtecan + AZD5305 • Sub-study 1d: datopotamab deruxtecan + *Imfinzi* + AZD5305 • Sub-study 2 (gastric cancer); • Sub-study 2a: datopotamab deruxtecan + capecitabine • Sub-study 2b: datopotamab deruxtecan + 5-fluorouracil • Sub-study 2c: datopotamab deruxtecan + chemotherapy (capecitabine or 5-FU) + nivolumab • Sub-study 3 (mCRPC); • Sub-study 3a: datopotamab deruxtecan • Sub-study 3b: datopotamab deruxtecan + AZD5305 • Sub-study 4 (ovarian cancer) • Sub-study 4a: datopotamab deruxtecan • Sub-study 4b • Arm1: datopotamab deruxtecan + carboplatin • Arm2: datopotamab deruxtecan + AZD5305 • Sub-study 5 (CRC) • Sub-study 5a: datopotamab deruxtecan • Sub-study 5b • Arm 1: datopotamab deruxtecan + 5-FU + leucovorin + bevacizumab • Arm 2: datopotamab deruxtecan + capecitabine + bevacizumab • Sub-study 6 (bladder) • Arm 1: 1L cis-ineligible/2L datopotamab deruxtecan + volrustomig • Arm 2: 1L cis-ineligible/2L datopotamab deruxtecan + rilvegostomig • Sub-study 7 (BTC) • Arm 7a: TROP2+ 2L+ datopotamab deruxtecan	• Primary endpoints: ORR and safety	• FPCD: Q3 2022 • Data anticipated: >2024

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Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II TROPION-PanTumor02 NCT05460273 Partnered (Daiichi Sankyo)	NSCLC and TNBC and other solid tumours in Chinese patients	119	• Single-arm, multi-cohort study with no blinding • datopotamab deruxtecan • China only	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, BOR, TTR PFS and OS	• FPCD: Q3 2022 • LPCD: Q2 2023 • Data anticipated: H2 2023
Phase I TROPION-PanTumor01 NCT03401385 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+ breast cancer	890	• Open-label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan • US and Japan	• Primary endpoints: DLT and safety • Secondary endpoints: PK parameters, anti-tumour activity and ADA	• FPCD: Q1 2018 • Data anticipated: H2 2024

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Appendix: Glossary.

ceralasertib (AZD6738, ATR inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III LATIFY NCT05450692	Post-IO NSCLC	580	• Double-arm randomised: • Arm 1: ceralasertib + *Imfinzi* • Arm 2: docetaxel	• Primary endpoint: OS • Secondary endpoint: PFS, ORR, DoR, TTR, DCR, PFS2 and TTD	• FPCD: Q4 2022 • Data anticipated: >2024
Phase II MONETTE NCT05061134	2L+ post-IO melanoma	195	• Double-armed randomised and biopsy sub-study • Arm 1: ceralasertib + *Imfinzi* • Arm 2: ceralasertib • Arm 3: ceralasertib (biopsy sub-study)	• Primary endpoint: ORR • Secondary endpoints: DoR, TTR, PFS, OS, safety and biomarkers	• FPCD: Q3 2022 • Data anticipated: H1 2024
Phase I/II NCT02264678	Solid tumours	330	• Module 1: ceralasertib + carboplatin • Module 2: ceralasertib dose escalation, ceralasertib + *Lynparza* • Module 3: ceralasertib + *Imfinzi* • Module 4: ceralasertib monotherapy + *Lynparza* + *Imfinzi* (food effect/QT) • Module 5: ceralasertib + AZD5305 • Global trial – North America, Europe and South Korea	• Primary endpoints: safety and tolerability, efficacy and PK parameters	• FPCD: Q4 2014 • Data anticipated: >2024

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Appendix: Glossary.

Oncology: early-stage development

AZD0171 (anti-LIF mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04999969**	1L metastatic pancreatic ductal adenocarcinoma	115	• Open-label, non-randomised trial • AZD0171 + *Imfinzi* + gemcitabine, nab-paclitaxel	• Primary endpoints: safety, OS at 12 months • Secondary endpoints: ORR, DoR, PFS	• FPCD: Q1 2022 • Data anticipated: H2 2024

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Appendix: Glossary.

AZD0466 (Bcl2/xL inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **NCT04865419**	Advanced haematologic malignancies	141	• Module 1: • Part A: dose escalation (AZD0466) • Part B: dose expansion (AZD0466) • Module 2: DDI trial • AZD0466 with voriconazole	• Primary endpoint: safety • Secondary endpoint: PK parameters	• FPCD: Q2 2021 • Trial discontinued based on benefit-risk profile assessment
Phase I/II **NCT05205161**	Advanced non-Hodgekin lymphoma	50	• Part A: dose escalation • Part B: dose expansion Arm 1: R/R MCL • Part B: dose expansion Arm 2: R/R FL or MZL • Part B: dose expansion Arm 3: R/R DLBCL	• Primary endpoint (Part A): safety • Primary endpoint (Part B): ORR	• FPCD: Q3 2022 • Trial discontinued based on benefit-risk profile assessment

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Appendix: Glossary.

AZD1390 (ATM inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03423628**	Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients	120	• Open-label trial • Arm 1: recurrent GBM, AZD1390 + RT in dose escalation cohorts • Arm 3: primary GBM, AZD1390 + RT in dose escalation cohorts	• Primary endpoints: safety, tolerability and MTD • Secondary endpoints: PK parameters and preliminary assessment of anti-tumour activity	• FPCD: Q2 2018 • Data anticipated: H2 2024

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Appendix: Glossary.

AZD4573 (CDK9 inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT05140382**	R/R Peripheral T-cell lymphoma and R/R classical Hodgkins lymphoma	79	• Open label, non-randomised modular dose confirmation and expansion trial in patients with R/R PTCL or cHL • Module 1: AZD4573 monotherapy • Cohort 1: PTCL, all comers (excluding NKTCL) • Cohort 3: cHL • i.v. route of administration • Global trial – 9 countries	• Primary endpoint: efficacy • Secondary endpoints: safety and PK parameters	• FPCD: Q4 2021 • Trial discontinued due to strategic portfolio prioritisation
Phase I/II **NCT04630756**	R/R haematologic malignancies	37	• Open label, non-randomised trial • Module 1 Part A: dose setting • AZD4573 + *Calquence* (100mg BID) combination in DLBCL, all comers; ramp-up across 3 dose levels • Module 1 Part B: dose expansion • AZD4573 + *Calquence* (100mg BID) combination in GCB and non-GCB DLBCL • Module 2 Part A: dose confirmation • AZD4573 monotherapy window followed by AZD4573 + acalabrutinib in patients with R/R MCL • i.v. route of administration • Global trial – 10 countries	• Primary endpoint (Part A): safety • Primary endpoint (Part B): ORR • Secondary endpoints: safety, PK parameters and anti-tumour activity	• FPCD: Q1 2021 • Trial discontinued due to strategic portfolio prioritisation
Phase I **NCT03263637**	R/R haematologic malignancies	44	• Arm 1: dose escalation in haematological malignancies excluding AML/ALL/high-risk MDS/CMML/CLL • Arm 2: dose escalation in R/R AML, ALL, high-risk MDS, CMML, CLL and Richter's syndrome • i.v. route of administration • Global trial – Netherlands, UK and Germany	• Primary endpoints: safety and PK parameters • Secondary endpoint: efficacy	• FPCD: Q4 2017 • LPCD: Q3 2021 • Trial discontinued due to strategic portfolio prioritisation

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Appendix: Glossary.

AZD5305 (PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **PETRA** **NCT04644068**	Advanced, metastatic HER2- breast cancer (BRCAm, PALB2m or RAD51C/Dm); advanced, metastatic TNBC; PSR ovarian cancer (BRCAm, PALB2m or RAD51C/Dm); PSR ovarian cancer (HRD+); prostate cancer (mCRPC, BRCAm); prostate cancer (mCRPC, HRRm); pancreatic cancer	559	• Modular, open-label, multi-centre dose escalation and expansion trial • Module 1: AZD5305 • Module 2: AZD5305 + paclitaxel • Module 3: AZD5305 + carboplatin +/- paclitaxel • Module 4: AZD5305 + *Enhertu* • Module 5: AZD5305 + datopotamab deruxtecan	• Primary endpoints: safety and tolerability, PK parameters • Secondary endpoint: efficacy	• FPCD: Q4 2020 • Data anticipated: >2024
Phase I/IIa **PETRANHA** **NCT05367440**	Metastatic prostate cancer	172	• Multi-arm, open-label, non-randomised, multi-centre trial of AZD5305 in combination with physicians' choice new hormonal agents in patients with metastatic • prostate cancer • Arm 1: AZD5305 + enzalutamide • Arm 2: AZD5305 + abiraterone acetate • Arm 3: AZD5305 + darolutamide	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and efficacy	• FPCD: Q2 2022 • Data anticipated: >2024
Phase I **NCT05573724**	Locally advanced, unresectable or metastatic solid tumours	14	• Part A: to assess the effect of multiple doses of itraconazole on the single-dose PK parameters of AZD5305 which will last up to 13 days and follows a non-randomised, open-label, 2 intervention design • Part B: option to continue with AZD5305 monotherapy after completing Part A and whilst obtaining clinical benefit	• Primary endpoint: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q4 2022 • LPCD: Q2 2023 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

AZD5335 (anti-FRα TOP1i ADC)

Solid tumours, ovarian cancer, lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II FONTANA NCT05797168	Advanced solid tumour malignancies	150	• Module 1: AZD5335 monotherapy • Module 2: AZD5335 in combination with AZD5305	• Primary endpoints: safety and tolerability • Secondary endpoints: efficacy and PK parameters	• Data anticipated: H2 2024

As of 28 July 2023.
Appendix: Glossary.

AZD8205 (B7H4 ADC)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **NCT05123482**	Breast cancer, biliary tract cancer, ovarian cancer, endometrial cancer	280	• Open-label, non-randomised dose-escalation, and randomised/non-randomised dose-expansion trial in monotherapy • AZD8205	• Primary endpoints: AE, SAE, DLTs, changes in lab and preliminary efficacy parameters • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

AZD9574 (PARP1-sel BBB inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa CERTIS-1 NCT05417594	Advanced solid malignancies	195	• Modular, open-label, multi-centre dose escalation and expansion trial • Module 1: AZD9574 monotherapy • Module 2: AZD9574 + temozolomide	• Primary endpoints: safety and tolerability of AZD9574 as monotherapy and in combination with anti-cancer agents • Secondary endpoints: PK parameters and efficacy of AZD9574 as monotherapy and in combination with anti-cancer agents	• FPCD: Q3 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

AZD9592 (EGFR-cMET TOP1i ADC)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **EGRET** **NCT05647122**	Advanced solid tumours including NSCLC and HNSCC	108	• Escalation phase, open-label, multi-centre trial • AZD9592 • AZD9592 + *Tagrisso* • Expansion phase, open-label, multi-centre trial • AZD9592 • AZD9592 + *Tagrisso*	• Primary endpoints (escalation): safety and tolerability • Primary endpoints (expansion): safety and tolerability, anti-tumour activity • Secondary endpoints (escalation): PK parameters, immunogenicity, anti-tumour activity • Secondary endpoints (expansion): PK parameters and immunogenicity	• FPCD: Q1 2023 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

IPH5201 (CD39 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04261075** **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 • Part 1: IPH5201 monotherapy dose escalation to MTD • Part 2: IPH5201 + *Imfinzi* dose escalation to MTD • Part 3: IPH5201 + *Imfinzi* + oleclumab dose escalation to MTD • Route of administration: i.v. • Global trial – US and EU	• Primary endpoints: AE, SAE and DLT • Secondary endpoints: OR, DC, PK parameters and ADA	• FPCD: Q1 2020 • LPCD: Q2 2022 • Data readout: Q1 2023

As of 28 July 2023.
Appendix: Glossary.

oleclumab (CD73 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II **NCT03611556**	Pancreatic 1L and 2L with prior gemcitabine-based chemotherapy	339	• Arm A1: gemcitabine and nab paclitaxel i.v. • Arm A2: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. • Arm A3: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. + *Imfinzi* i.v. • Arm B1: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. • Arm B2: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. • Arm B3: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. + *Imfinzi* i.v. • Global trial – US, Norway, Spain and Australia	• Primary endpoints: safety and anti-tumour activity • Secondary endpoints: PFS, PK parameters, immunogenicity, safety and anti-tumour activity	• FPCD: Q2 2018 • LPCD: Q3 2022 • Data readout: Q1 2023

As of 28 July 2023.
Appendix: Glossary.

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

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **ARTEMIDE-01** **NCT04995523** **Partnered (Compugen)**	NSCLC	192	• Open-label, dose escalation and dose expansion trial • Part A: dose escalation in CPI-experienced NSCLC patients with rilvegostimig i.v. monotherapy • Part B: dose expansion in CPI-experienced NSCLC patients with rilvegostimig i.v. monotherapy • Part C: dose expansion in CPI-naive NSCLC patients with rilvegostimig i.v. monotherapy • Part D: randomised dose expansion in CPI-naive NSCLC patients with rilvegostimig i.v. monotherapy • Global trial – Europe, Australia, Taiwan, South Korea, Japan, China, Brazil and North America	• Primary endpoints (Part A): safety, RP2D and MTD • Primary endpoints (Part B): safety and efficacy (ORR) • Primary endpoints (Part C): safety and efficacy (ORR) • Primary endpoints (Part D): safety and efficacy (ORR) • Secondary endpoints: PK parameters, PD (receptor occupancy), efficacy (DCR, DoR, DRR, PFS)	• FPCD: Q4 2021 • Data anticipated: H1 2024
Phase IIb **GEMINI-GC** **NCT05702229** **Partnered (Compugen)**	Gastric cancer	80	• Open-label gastric platform study • Sub-study 1: volrustomig combined with XELOX or volrustomig combined with FOLFOX • Sub-study 2: rilvegostomig combined with XELOX or rilvegostomig combined with FOLFOX	• Primary endpoints: safety and efficacy (ORR and PFS6) • Secondary endpoints: DoR, OS, PK, ADA and safety	• FPCD: Q1 2023 • Data anticipated: >2024
Phase IIb **GEMINI-HPB** **NCT05775159** **Partnered (Compugen)**	HCC, BTC	180	• Open-label hepatobiliary platform study • HCC sub-study: • Cohort 1A: volrustomig monotherapy • Cohort 1B: volrustomig combination with bevacizumab • Cohort 1C: volrustomig combination with lenvatinib • BTC sub-study: • Cohort 2A: rilvegostomig combination with gemcitabine and cisplatin • Cohort 2B: volrustomig combination with gemcitabine and cisplatin	• Primary endpoints (HCC sub-study): safety and efficacy (ORR) • Primary endpoints (BTC sub-study): safety and efficacy (PFS6) • Secondary endpoints: DoR, OS, PK and ADA	• FPCD: Q2 2023 • Data anticipated: H2 2024

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Appendix: Glossary.

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

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT04931654**	NSCLC, other tumours	152	• Open-label, non-randomised dose-escalation and dose-expansion trial • Part A: dose escalation in post-IO NSCLC patients with sabestomig i.v. monotherapy • Part B: dose expansion in post-IO and IO-naïve NSCLC patients with sabestomig i.v. monotherapy • Global trial	• Primary endpoints: AE, SAE, DLTs and ORR • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK parameters, ADA and ctDNA	• FPCD: Q4 2021 • Data anticipated: >2024
Phase I/II **NCT05216835**	R/R classical Hodgkin lymphoma	180	• Cohort A: dose escalation where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive sabestomig • Cohort B1: dose expansion where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive sabestomig once the recommended Phase II dose (RP2D) has been determined • Cohort B2: dose expansion where patients with anti-PD-1/PD-L1 naïve R/R cHL will receive sabestomig once the RP2D has been determined	• Primary endpoints (Cohort A): AE and DLTs • Primary endpoints (Cohort B1): AE and ORR • Primary endpoints (Cohort B2): AE and CRR • Secondary endpoints (Cohort A): CRR, ORR, DoR, DoCR, PFS, OS, ADA and PK parameters • Secondary endpoints (Cohort B1 and B2): DoR, DoCR, PFS, OS, ADA and PK parameters	• FPCD: Q2 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

TNB-486 (CD19/CD3 next-generation bispecific T-cell engager)

Haematologic malignancies

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04594642**	R/R B-cell non-Hodgkin lymphoma	116	• Multi-centre, Phase I, open-label, dose-escalation and expansion trial • TNB-486	• Primary endpoints: safety and tolerability, PK parameters • Secondary endpoints: clinical activity of monotherapy TNB-486, anti-drug anitbody titers for monotherapy TNB-486	• FPCD: Q1 2021 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

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

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib NCT04522323	Advanced renal cell carcinoma	70	• Open-label, dose escalation and dose expansion trial • Arm 1: volrustomig and axitinib • Arm 2: volrustomig and lenvatanib	• Primary endpoints (escalation): safety, MTD, RP2D, tolerability and anti-tumour activity of combination (ORR) • Secondary endpoints: PK parameters, ADA and anti-tumour activity (PFS, OR, DoR, DCR, TTR, OS)	• FPCD: Q3 2020 • Data anticipated: >2024
Phase I NCT03530397	Advanced solid tumours	396	• Open-label, dose-escalation and dose-expansion trial • Dose escalation: volrustomig i.v. • Dose expansion: volrustomig i.v. as monotherapy and in combination with chemotherapy • Arm 1: volrustomig i.v. • Arm 2: volrustomig i.v., pemetrexed and carboplatin • Arm 3: pembrolizumab, pemetrexed and carboplatin • Arm 4: volrustomig i.v., taxane (paclitaxel or nab-paclitaxel) and carboplatin	• Primary endpoints (escalation): safety and tolerability, MTD, OBD and HPDD • Primary endpoint (expansion): antitumour activity based on ORR • Secondary endpoints: PK parameters, ADA, tumoural baseline PD-L1, anti-tumour activity (OR, DoR, DCR, PFS, OS)	• FPCD: Q2 2018 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

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

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase Ia/Ib	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 • NT-125	• Primary endpoints (Phase Ia): incidence of AEs defined as DLTs • Primary endpoints (Phase Ib): ORR per RECIST v.1.1 • Secondary endpoints (Phase Ia): percentage of pre-screened and enrolled subjects that receive treatment • Secondary endpoints (Phase Ib): percentage change tumour size, best percentage change tumor size, DoR, clinical benefit rate, TTP, PFS and OS	• FPCD: Q2 2023 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

BioPharmaceuticals: approved medicines and late-stage pipeline

Andexxa (anti-factor Xa reversal)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase IV **I8-513 (post-launch)** **NCT03661528**	Acute intracranial haemorrhage	1200	• Arm 1: *Andexxa* • Arm 2: usual care • Global trial	• Primary endpoint: proportion of patients with good or excellent haemostatic efficacy as rated by an independent adjudication committee • Secondary endpoint: change from baseline in anti-fXa activity	• FPCD: Q2 2019 • Data anticipated: H2 2023
Phase II **19-515** **NCT04233073**	Urgent surgery	10	• Arm 1: *Andexxa*	• Primary endpoint: proportion of patients with good or excellent intraoperative haemostatic efficacy as determined by the surgeon's assessment and confirmed by an independent adjudication committee • Secondary endpoint: percent change from baseline in anti-factor Xa activity	• FPCD: Q2 2021 • LPCD: Q1 2022 • Data readout: Q4 2022

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Appendix: Glossary.

Farxiga (SGLT2 inhibitor)

Heart failure and chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DELIVER** **NCT03619213**	CHF patients with HFpEF	6263	• Arm 1: *Farxiga* 10mg QD • Arm 2: placebo • Global trial – 21 countries	• Primary endpoint: time to the first occurrence of any of the components of the composite (CV death or hospitalisation for HF or an urgent HF visit)	• FPCD: Q4 2018 • LPCD: Q1 2022 • Data readout: Q2 2022 • Primary endpoint met
Phase III **DAPA-MI** **NCT04564742**	Myocardial infarction	6400	• Arm 1: *Farxiga* 10mg QD • Arm 2: placebo • Global trial – 2 countries	• Primary endpoint: time to the first occurrence of any of the components of the composite (hospitalisation for HF or CV death)	• FPCD: Q4 2020 • Data anticipated: H2 2023
Phase I **NCT04856007**	Healthy Chinese volunteers	80	• Arm 1: *Farxiga* 5mg + metformin 500mg XR • Arm 2: *Farxiga*/metformin XR FDC 5/500mg • Arm 3: *Farxiga* 10mg + metformin 1000mg XR • Arm 4: *Farxiga*/metformin XR FDC 10/1000mg • China only	• Primary endpoint: plasma AUCinf, AUClast and Cmax of Farxiga and metformin	• FPCD: Q2 2021 • LPCD: Q2 2021 • Data readout: Q4 2021
Phase I **NCT05266404**	Healthy volunteers	46	• Arm 1: *Farxiga* 10mg + sitagliptin 100mg • Arm 2: *Farxiga*/sitagliptin FDC 10/100mg • Germany only	• Primary endpoint: AUCinf, AUClast and Cmax of Farxiga and sitagliptin	• FPCD: Q2 2022 • LPCD: Q2 2022 • Data readout: Q4 2022

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Appendix: Glossary.

Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb **DIALIZE China** **NCT04217590**	ESRD with hyperkalaemia and on stable haemodialysis	134	• Arm 1: *Lokelma* 5g QD for 8 weeks on non-dialysis days with option to uptitrate to 10g and 15g QD • Arm 2: placebo QD for 8 weeks on non-dialysis days • China only	• Primary endpoint: proportion of patients who maintain a pre-dialysis serum K between 4.0 and 5.0 mmol/L on 3 out of 4 dialysis treatments following the long interdialytic interval	• FPCD: Q4 2020 • LPCD: Q3 2021 • Data readout: Q1 2022 • Primary endpoint met
Phase III **DIALIZE-Outcomes** **NCT04847232**	Recurrent hyperkalaemia on chronic haemodialysis	2800	• Arm 1: *Lokelma* 5g to 15g QD for 4 weeks on non-dialysis days; thereafter, adjusted monthly • Arm 2: placebo QD • Global trial – 26 countries	• Primary endpoint: time to first occurrence of SCD, stroke or hospitalisation, intervention or ED visit due to arrhythmia	• FPCD: Q3 2021 • Data anticipated: >2024
Phase III **STABILIZE-CKD** **NCT05056727**	Patients with CKD and hyperkalaemia or at risk of hyperkalaemia	1360	• Open-label *Lokelma* (10g TID or 5g QD) for up to 72 hours, followed by 3 months open-label treatment with *Lokelma* (5g QOD to 15g QD) and uptitration of lisinopril or valsartan; thereafter, patients are randomised to a 24 month treatment: • Arm 1: *Lokelma* (5g QOD to 15g QD) and lisinopril or valsartan • Arm 2: placebo and lisinopril or valsartan • Global trial – 20 countries	• Primary endpoints: total slope (eGFR measurements starting at randomisation) and chronic slope (eGFR measurements starting at 12 weeks after randomisation)	• FPCD: Q4 2021 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III MATTERHORN NCT03263091 Partnered (FibroGen)	Anaemia in lower-risk MDS patients	184	• Open-label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo • Global trial	• Primary endpoint: proportion of patients achieving transfusion independence	• FPCD: Q3 2017 • Data readout: Q2 2023 • Primary endpoint not met
Phase II/III NCT03303066 Partnered (FibroGen)	Anaemia in lower-risk MDS patients	43	• Open-label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo • China only	• Primary endpoint: haemoglobin response	• FPCD: Q2 2018 • LPCD: Q1 2023 • Data readout: Q2 2023 • Primary endpoint met

As of 28 July 2023.
Appendix: Glossary.

baxdrostat (selective aldosterone synthase inhibitor)

Hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase II **SPARK** **NCT04605549**	Patients with primary aldosteronism	18	• Arm 1: baxdrostat 2-8mg QD • 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 • 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 • Data anticipated: >2024
Phase II **HALO-OLE** **NCT05459688**	Patients with hypertension who have completed CIN-107-124	175	• Arm 1: baxdrostat 2mg QD • US only	• Primary endpoints: safety and tolerability	• FPCD: Q2 2022 • Data anticipated: H2 2023
Phase II **FigHTN** **NCT05432167**	Patients with uncontrolled hypertension and CKD	300	• Arm 1: baxdrostat (low dose) • Arm 2: baxdrostat (high dose) • Arm 3: placebo • US only	• Primary endpoint: change from baseline in mean seated systolic blood pressure vs. placebo at Week 26 • Secondary endpoint: to evaluate the treatment effect on SBP at Week 26 by dosing strategy	• FPCD: Q2 2022 • Data anticipated: H1 2024

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Appendix: Glossary.

eplontersen (ligand-conjugated antisense)

ATTR

Trial	Population	Patients	Design	Endpoints	Status
Phase III CARDIO-TTRansform NCT04136171 Partnered (Ionis Pharmaceuticals, Inc.)	Hereditary or wild-type transthyretin-mediated amyloid cardiomyopathy (ATTR-CM)	1400	• Arm 1: eplontersen s.c. • Arm 2: placebo	• Primary endpoints: composite outcome of CV mortality and recurrent CV clinical events at Week 140 • Secondary endpoints: 6MWT, KCCQ, CV events and CV mortality	• FPCD: Q1 2020 • Data anticipated: >2024
Phase III NEURO-TTRansform NCT04136184 Partnered (Ionis Pharmaceuticals, Inc.)	Hereditary transthyretin-mediated amyloid polyneuropathy (ATTRv-PN)	168	• Arm 1: eplontersen s.c. • Arm 2: inotersen s.c.	• Primary endpoints (at Week 35): change from baseline in mNIS+7 and percent change from baseline in TTR concentration • Secondary endpoint (Week 35): changes from baseline in Norfolk QOL • 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 • Data readout: Q2 2022 • Co-primary endpoints met

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Appendix: Glossary.

mitiperstat (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb/III ENDEAVOR NCT04986202	HFpEF	1485	• Randomised, double-blind • Arm 1: 2.5mg mitiperstat • Arm 2: 5mg mitiperstat • Arm 3: placebo • Global trial	• Primary endpoints: safety and efficacy	• FPCD: Q3 2021 • Data anticipated: H1 2024

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Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

Diabetic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04170543**	Adult patients with diabetic kidney disease	581	• Arm 1: tozorakimab dose 1 + *Farxiga* • Arm 2: tozorakimab dose 2 + *Farxiga* • Arm 3: tozorakimab dose 3 + *Farxiga* • Arm 4: tozorakimab dose 4 + *Farxiga* • Arm 5: placebo + *Farxiga* • Global trial – US, Canada, Japan and additional countries	• Primary endpoint: change from baseline in UACR compared to placebo at 24 weeks • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2019 • LPCD: Q3 2022 • Data anticipated: H2 2023

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Appendix: Glossary.

zibotentan (endothelin receptor antagonist)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05505162**	Healthy female volunteers of non-childbearing potential	24	• Open-label, single-sequence , single-centre trial • US only	• Primary endpoints: PK parameters	• FPCD: Q3 2022 • Data readout: Q2 2023

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Appendix: Glossary.

zibotentan (endothelin receptor antagonist)

Liver Cirrhosis with Features of portal hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ZEAL** **NCT05516498**	Part A: participants with Child-Pugh A cirrhosis with features of portal hypertension and with no history of decompensation events Part B: participants with a broader range of Child-Pugh A and Child-Pugh B cirrhosis with more severe disease	140	• Phase IIa/b multi-centre, randomised, double-blind, placebo-controlled, parallel group dose-ranging trial • Part A Arm 1: placebo • Part A Arm 2: zibotentan dose B + *Farxiga* • Part B Arm 1: placebo • Part B Arm 2: placebo + *Farxiga* • Part B Arm 3: zibotentan dose A + *Farxiga* • Part B Arm 4: zibotentan dose B + *Farxiga* • Part B Arm 5: zibotentan dose C + *Farxiga* • Global trial	• Primary endpoint (Part A): absolute change in HVPG from baseline to Week 6 comparing zibotentan and *Farxiga* in combination vs. placebo • Primary endpoint (Part B): HVPG response from baseline to Week 6 comparing zibotentan and *Farxiga* in combination and *Farxiga* monotherapy vs. placebo	• FPCD: Q4 2022 • Data anticipated: H2 2024

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Appendix: Glossary.

Airsupra (PT027, SABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III MANDALA NCT03769090 Managed by Avillion (Avillion)	Moderate to severe asthma	3132	• Randomised, double-blind, multi-centre, parallel group • Treatments: minimum 24-week treatment period • BDA (budesonide albuterol) MDI 80/180µg prn • BDA MDI 160/180µg prn • AS (albuterol sulphate) MDI 180µg prn • Global trial	• Primary endpoint: time to first severe asthma exacerbation • Secondary endpoints: severe exacerbation rate (annualised); total corticosteroid exposure over the treatment period; Asthma Control Questionnaire -5 change from baseline and responder analysis at Week 24; Asthma Quality of Life questionnaire for 12 years and older/Paediatric Asthma Quality of Life questionnaire change from baseline and responder analysis at Week 24	• FPCD: Q4 2018 • LPCD: Q1 2021 • Data readout: Q3 2021 • Primary endpoint met
Phase III DENALI NCT03847896 Managed by Avillion (Avillion)	Mild to moderate asthma	1001	• Randomised, double-blind, multi-centre and parallel-group • Treatments: 12-week treatment period • BDA MDI 80/180µg QID • BDA MDI 160/180µg QID • BD MDI 160µg QID • AS MDI 180µg QID • placebo MDI QID • Global trial	• Dual primary endpoints: change from baseline in FEV1 AUC0-6 hours over 12 weeks; change from baseline in trough FEV1 at Week 12	• FPCD: Q2 2019 • LPCD: Q2 2021 • Data readout: Q3 2021 • Dual primary endpoints met
Phase III TYREE NCT04234464 Managed by Avillion (Avillion)	Asthma with exercise induced bronchoconstriction	60	• Randomised, double-blind, multi-centre crossover • Treatments: single-dose • BDA MDI 160/180µg • placebo MDI QID • US only	• Primary endpoint: maximum percentage fall from post-dose, pre-exercise baseline in FEV1 observed up to 60 minutes post-exercise challenge	• FPCD: Q1 2020 • LPCD: Q3 2020 • Data readout: Q4 2020 • Primary endpoint met

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Appendix: Glossary.

Breztri/ Trixeo (LAMA/LABA/ICS)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KALOS** **NCT04609878**	Severe asthma	2200	• Randomised, double-blind, double-dummy, parallel group and multi-centre trial • Treatments (24- to 52-week variable length) • BGF 320/28.8/9.6µg BID MDI • BGF 320/14.4/9.6µg BID MDI • BFF 320/9.6µg BID MDI • *Symbicort* 320/9µg BID pMDI • Global trial	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 • Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data anticipated: >2024
Phase III **LOGOS** **NCT04609904**	Severe asthma	2200	• Randomised, double-blind, double dummy, parallel group and multi-centre trial • Treatments (24- to 52-week variable length) • BGF 320/28.8/9.6µg BID MDI • BGF 320/14.4/9.6µg BID MDI • BFF 320/9.6µg BID MDI • *Symbicort* 320/9µg BID pMDI • Global trial	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24 • Secondary endpoint: change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data anticipated: >2024
Phase III **VATHOS** **NCT05202262**	Moderate asthma	630	• Randomised, double-blind, parallel group, multi-centre trial • Treatments (24-week) • BFF 320/9.6µg BID MDI • BFF 160/9.6µg BID MDI • BD 320µg BID MDI • Open-label *Symbicort* TBH 320/9µg BID • Global trial	• Primary endpoint: change from baseline in FEV1 AUC0-3 at Week 24	• FPCD: Q1 2022 • Data anticipated: H2 2024
Phase III **LITHOS** **NCT05755906**	Mild to moderate asthma	340	• Randomised, double-blind, parallel group and multi-centre • Treatments (12-week) • BFF 160/9.6µg BID MDI • BD 160µg BID MDI • 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 • Data anticipated: H2 2024

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Appendix: Glossary.

Breztri/ Trixeo (LAMA/LABA/ICS)

COPD

Trial	Population	Patients	Design	Endpoints	Status
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 • BGF MDI HFO 160/7.2/4.8µg (2 inhalations BID) • BGF 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 • Data anticipated: H2 2024
Phase I NCT05477108	Healthy volunteers	108	• Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way cross-over trial • BGF MDI HFO 160/7.2/4.8µg (single dose of 4 inhalations) • BGF MDI HFA 160/7.2/4.8µg (single dose of 4 inhalations)	• Primary endpoint: AUCinf, AUClast and Cmax	• FPCD: Q3 2022 • Data anticipated: H2 2023
Phase I NCT05569421	Healthy volunteers	108	• Randomised, double-blind, single-dose, single-centre, partial-replicate, 3-way cross-over trial • BGF MDI HFO 160/7.2/4.8µg (single dose of 4 inhalations) • BGF MDI HFA 160/7.2/4.8µg (single dose of 4 inhalations)	• Primary endpoint: AUCinf, AUClast and Cmax	• FPCD: Q4 2022 • Data anticipated: H1 2024

As of 28 July 2023.
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 • Arm 1: HFO MDI; 6 inhalations BID for 7 days • Arm 2: HFA MDI; 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 • Secondary endpoint: change from baseline in MCC at 3 hours following inhalation of 99m technetium sulfur colloid and gamma camera imaging	• FPCD: Q2 2023 • Data anticipated: H2 2024
Phase III NCT05850494	Well-controlled or partially-controlled asthma	52	• Randomised, mulit-centre double-blind, single-dose crossover trial • Arm 1: HFO propellant only MDI; 4 inhalations per dose • Arm 2: HFA propellant only MDI; 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 • Data anticipated: H1 2024

As of 28 July 2023.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Dermatology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **FJORD** **NCT04612790**	Patients with symptomatic (newly diagnosed or relapsing) bullous pemphigoid	120	• Double-blind, open-label trial • Arm 1: *Fasenra* • Arm 2: placebo • 36-week • Global trial	• Primary endpoint: proportion of patients with complete sustained (≥2 months) remission off OCS at 36 weeks	• FPCD: Q2 2021 • Trial discontinued for futility

As of 28 July 2023.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Nasal polyposis and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III OSTRO 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. • Arm 2: placebo s.c. • 56-week trial • Global trial – 8 countries	• Primary endpoint: effect of *Fasenra* on nasal polyp burden and on patient reported nasal blockage	• FPCD: Q1 2018 • LPCD: Q2 2019 • Data readout: Q3 2020 • Co-primary endpoints met
Phase III ORCHID NCT04157335	Patients with eosinophilic chronic rhinosinusitis with severe nasal polyposis; age 18 to 75 years	276	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo Q8W s.c. • 56-week trial • Global trial – 10 countries	• Primary endpoints: change in endoscopic total nasal polyp score and change in mean nasal blockage score	• FPCD: Q4 2019 • Data anticipated: H2 2024
Phase III MANDARA 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. • Arm 2: mepolizumab 300mg Q4W s.c. • 52-week trial with a minimum 1-year open label extension • 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 • Data anticipated: H2 2023
Phase III NATRON 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	120	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 24-week trial with a minimum 1-year open label extension • Global trial – 9 to 12 countries	• Primary endpoint: time to first HES worsening/flare	• FPCD: Q3 2020 • Data anticipated: H1 2024

As of 28 July 2023.
Appendix: Glossary.

Fasenra (IL-5R mAb)

Severe, uncontrolled asthma and COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III MIRACLE NCT03186209	Severe, uncontrolled asthma despite background controller medication, MD and HD ICS + LABA ± chronic OCS; age 12 to 75 years	695	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo s.c. • 56-week trial	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: pulmonary function, asthma symptoms and other asthma control metrics	• FPCD: Q4 2017 • LPCD: Q4 2021 • Data readout: Q1 2023 • Primary endpoint met
Phase III RESOLUTE 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	642	• Double-blind, placebo-controlled trial • Arm 1: *Fasenra* 100mg Q8W s.c. • Arm 2: placebo Q8W s.c. • 56-week treatment • Global trial – 26 countries	• Primary endpoint: annualised rate of moderate or severe exacerbations over 56 weeks	• FPCD: Q4 2019 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

Saphnelo (type I interferon receptor mAb)

Lupus (SLE/LN)

Trial	Population	Patients	Design	Endpoints	Status
Phase III TULIP-SC NCT04877691 Partnered (BMS)	Moderate to severe SLE	360	• Arm 1: *Saphnelo* s.c. • Arm 2: placebo s.c. • Global trial	• Primary endpoint: BICLA at Week 52	• FPCD: Q3 2021 • Data anticipated: >2024
Phase III AZALEA-SLE NCT04931563 Partnered (BMS)	Moderate to severe SLE patients	328	• Arm 1: 300mg *Saphnelo* i.v. Q4W • Arm 2: placebo i.v. Q4W • Asia only	• Primary endpoint: BICLA at Week 52	• FPCD: Q4 2021 • Data anticipated: >2024
Phase III IRIS NCT05138133 Partnered (BMS)	Active, proliferative LN	360	• Arm 1: *Saphnelo* i.v. • Arm 2: placebo i.v.	• Primary endpoint: CRR at Week 52	• FPCD: Q2 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

Tezspire (TSLP mAb)

CRSwNP, COPD and EoE

Trial	Population	Patients	Design	Endpoints	Status
Phase III WAYPOINT NCT04851964 Partnered (AMGEN)	Severe chronic rhinosinusitis with nasal polyps; age 18 years and older	400	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial • Global trial – 10 countries	• Co-primary endpoint: nasal polyp score and participant reported nasal congestion	• FPCD: Q2 2021 • Data anticipated: H2 2024
Phase III CROSSING NCT05583227 Partnered (AMGEN)	Adult and paediatric aged 12 years and older with eosinophilic esophagitis	360	• Arm 1: *Tezspire* s.c. low dose • Arm 2: *Tezspire* s.c. high dose • Arm 3: placebo • 52-week trial • 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 • Data anticipated: >2024
Phase IIa COURSE NCT04039113 Partnered (AMGEN)	Moderate to very severe COPD; age 40 to 80	338	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial • Global trial – 10 countries	• Primary endpoint: rate of moderate or severe COPD exacerbations	• FPCD: Q3 2019 • Data anticipated: H1 2024

As of 28 July 2023.
Appendix: Glossary.

Tezspire (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR NCT03347279 Partnered (AMGEN)	Severe asthma; age 12 to 80 years	1061	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial • Global trial – 18 countries	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6)	• FPCD: Q1 2018 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III DIRECTION NCT03927157 Partnered (AMGEN)	Severe asthma; age 18 to 80 years	396	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial • Regional trial (Asia) – 3 countries	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: change from baseline in pre-BD FEV1, asthma related QoL (AQLQ(S)+12) and asthma control (ACQ-6)	• FPCD: Q3 2019 • Data anticipated: H2 2024
Phase III SUNRISE NCT05398263 Partnered (AMGEN)	Severe asthma; age 18 to 80 years	207	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 28-week trial • Global trial – 10 countries	• Primary endpoint: categorised percent reduction from baseline in the daily maintenance OCS dose at Week 28 whilst maintaining asthma control	• FPCD: Q3 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

brazikumab (IL-23 inhibitor)

Inflammatory bowel disease (Crohn's disease, ulcerative colitis)

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NCT03961815**	Crohn's disease	161	• Open-label, long-term extension safety trial of brazikumab in participants with moderately to severely active Crohn's disease	• Primary endpoint: safety of long-term treatment with brazikumab (AEs, clinical laboratory values, vital signs and ECGs)	• FPCD: Q1 2020 • LPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation
Phase IIb/III **INTREPID** **NCT03759288**	Crohn's disease	928	• A 52-week, multi-centre, randomised, double-blind, placebo- and active-controlled, operationally seamless Phase IIb/III, parallel group trial to assess the efficacy and safety of brazikumab in participants with moderately to severely active Crohn's disease • Stage 1: • Arm 1: brazikumab high i.v. dose on Day 1, 29 and 57 + s.c. brazikumab on Day 85 and every 4 weeks through Week 48 • Arm 2: brazikumab low i.v. dose on Day 1, 29 and 57 s.c. brazikumab on Day 85 and every 4 weeks through Week 48 • Arm 3: placebo • Stage 2: • Arm 1: brazikumab high i.v. dose on Day 1, 29 and 57 + s.c. brazikumab on Day 85 and every 4 weeks through Week 48 • Arm 2: brazikumab low i.v. dose on Day 1, 29 and 57 s.c. brazikumab on Day 85 and every 4 weeks through Week 48 • Arm 3: adalimumab s.c. on Day 1, 15, 29 and every 2 weeks through Week 50	• Primary endpoint (Stage 1): CDAI remission at Week 12 • Stage 2 primary endpoints: Endoscopic response at week 52, Clinical remission at week 52 • Primary endpoints (Stage 2): endoscopic response at Week 52 and clinical remission at Week 52 • Secondary endpoints (Stage 1): endoscopic response at Week 12, clinical remission at Week 12, CDAI response at Week 12, response and remission at Week 52 •	• FPCD: Q4 2018 • LPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation
Phase II **EXPEDITION** **NCT03616821**	Ulcerative colitis	256	• A 54-week, multi-centre, randomised, double-blind, placebo-controlled, parallel-group trial to assess the efficacy and safety of brazikumab in participants with moderately to severely active ulcerative colitis • Arm 1: brazikumab dose 1 i.v. on Day 1, 15 and 43 + s.c. brazikumab from Day 71 and every 4 weeks • Arm 2: brazikumab dose 2 i.v. on day 1, 15 and 43 + s.c. brazikumab from Day 71 and every 4 weeks • Arm 3: placebo	• Primary endpoint: clinical remission at Week 10 • Secondary endpoint: sustained clinical remission at Week 10 and 54	• FPCD: Q3 2018 • LPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation

As of 28 July 2023.
Appendix: Glossary.

brazikumab (IL-23 inhibitor)

Inflammatory bowel disease (Crohn's disease, ulcerative colitis)

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04277546	Ulcerative colitis	165	• Open-label, long-term extension safety trial of brazikumab in participants with moderately to severely active ulcerative colitis	• Primary endpoint: safety of long-term treatment with brazikumab (AEs, clinical laboratory values, vital signs and ECGs)	• FPCD: Q1 2020 • LPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation
Phase I NCT05033431	Healthy volunteers	48	• Open-label trial to evaluate the PK, safety and tolerability of a single dose of brazikumab administered by i.v. infusion and s.c. injection in healthy Chinese and Caucasian participants	• Primary endpoint: PK parameters (Cmax, AUCinf, AUClast and AUC0-28d)	• FPCD: Q4 2021 • LPCD: Q4 2022 • Trial discontinued due to strategic portfolio prioritisation

As of 28 July 2023.
Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

Acute respiratory failure

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TILIA** **NCT05624450**	Adults hospitalised for viral lung infection requiring supplemental oxygen	2902	• Randomised, double-blind, placebo-controlled, parallel group • Arm 1: tozorakimab dose i.v. + SoC • Arm 2: placebo i.v. + SoC • Global trial – 36 countries	• Primary endpoint: progression to death or to invasive mechanical ventilation/extracorporeal membrane oxygenation • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

Atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **FRONTIER-3** **NCT04570657**	Adults with uncontrolled moderate to severe asthma	250	• Randomised, double-blind, placebo-controlled trial • Arm 1: tozorakimab dose 1 s.c. • Arm 2: tozorakimab dose 2 s.c. • Arm 3: placebo s.c. • Global trial – US, Argentina, Germany, Hungary, Poland, South Africa and UK	• Primary endpoint: change from baseline at Week 16 in FEV1 • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data readout: Q2 2023
Phase I **NCT05070312**	Healthy volunteers	36	• Randomised, double-blind, placebo-controlled, dose ascending trial • Arm 1: tozorakimab dose 1 s.c. • Arm 2: placebo s.c. • Arm 3: tozorakimab dose 2 s.c. • Arm 4: placebo s.c. • China only	• Primary endpoint: PK parameters • Secondary endpoint: immunogenicity	• FPCD: Q3 2021 • LPCD: Q4 2021 • Data readout: Q2 2022

As of 28 July 2023.
Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III OBERON NCT05166889	Adults with symptomatic COPD with a history of exacerbations	1272	• Randomised, double-blind, placebo-controlled, parallel-group • Treatment: 52-week • Arm 1: tozorakimab dose 1 s.c. + SoC • Arm 2: tozorakimab dose 2 s.c. + SoC • Arm 3: placebo s.c. + SoC • Global trial – 20 countries	• Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers), time to moderate to severe COPD exacerbation and change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q1 2022 • Data anticipated: >2024
Phase III TITANIA NCT05158387	Adults with symptomatic COPD with a history of exacerbations	1272	• Randomised, double-blind, placebo-controlled, parallel-group • Treatment: 52-week • Arm 1: tozorakimab dose 1 s.c. + SoC • Arm 2: tozorakimab dose 2 s.c. + SoC • Arm 3: placebo s.c.+ SoC • Global trial – 19 countries	• Primary endpoint: annualised rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualised rate of moderate to severe COPD exacerbations (former or current smokers), time to moderate to severe COPD exacerbation and change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q1 2022 • Data anticipated: >2024
Phase III PROSPERO NCT05742802	Subjects who completed either OBERON or TITANIA will be offered the opportunity to consent (adults with symtomatic COPD with a history of exacerbations)	2544	• Randomised, double-blind, placebo-contolled, parallel-group, long-term extension trial • Treatment: 52-weeks • Arm 1: tozorakimab dose 1 s.c. + SoC • Arm 2: tozorakimab dose 2 s.c. + SoC • Arm 3: placebo s.c. + SoC • Global trial – 38 countries	• Primary endpoint: time to first severe COPD exacerbation in primary population of former smokers over the treatment period incorporating both the predecessor studies and PROSPERO • Secondary endpoint: time to first severe COPD exacerbation in the overall population of current and former smokers	• FPCD: Q1 2023 • Data anticipated: >2024
Phase II NCT04631016	Adults with COPD and chronic bronchitis	137	• Randomised, double-blind, placebo-controlled, parallel-group, PoC trial • Arm 1: tozorakimab s.c. • Arm 2: placebo s.c. • Global trial – 15 countries	• Primary endpoint: change from baseline at Week 12 in FEV1 • Secondary endpoints: safety and other efficacy measures	• FPCD: Q1 2021 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ENDURE** **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 • Arm 1: *Evusheld*, dose regimen 1 • Arm 2: *Evusheld*, dose regimen 2 • US only	• Primary endpoints: safety and tolerability, incidence of ADA • Secondary endpoints: individual serum concentration; GMTs and GMFR in severe acute respiratory CoV-2 neutralizing antibodies	• FPCD: Q2 2022 • LPCD: Q3 2022 • Data anticipated: H1 2024
Phase I **NCT05166421**	Healthy adults; age ≥18 years	207	• Open-label, randomised, three-arm, single-dose trial • Arm 1: *Evusheld* administered as a single co-formulated dose (clonal cell line material) • Arm 2: *Evusheld* administered as two separate doses (clonal cell line material) • Arm 3: *Evusheld* administered as two separate doses (cell pool material) • *Evusheld* (1:1:1) • US only	• Primary endpoints: safety and PK parameters	• FPCD: Q1 2022 • Data anticipated: H2 2023
Phase I **TRUST** **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 • Cohort 1: pre-exposure prophylaxis • Cohort 2: mild-to-moderate COVID-19 • Cohort 3: severe COVID-19 • *Evusheld* • US only	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q1 2022 • Data anticipated: >2024

As of 28 July 2023.
Appendix: Glossary.

Beyfortus (nirsevimab, RSV mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELODY NCT03979313	Healthy infants (born 35 weeks 0 days or greater gestational age)	3000	• Randomised, double-blind, placebo-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: placebo i.m. • Global trial – 31 countries	• Primary endpoint: efficacy • Secondary endpoints: safety, PK parameters and ADA	• Data readout: Q3 2022 • FPCD: Q2 2021 (safety cohort) • LPCD: Q4 2021 (safety cohort) • Data readout: Q3 2022 (safety cohort) • Primary endpoint met • FPCD: Q3 2019 (efficacy cohort) • LPCD: Q1 2020 (efficacy cohort) • Data readout: Q2 2021 (efficacy cohort) • Primary endpoint met
Phase III CHIMES NCT05110261	Healthy infants (born 29 weeks 0 days or greater gestational age)	800	• Randomised, double-blind, placebo-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: placebo i.m. • China only	• Primary endpoints efficacy • Secondary endpoints: safety, PK parameters and ADA	• FPCD: Q4 2021 • Data anticipated: >2024
Phase IIb NCT02878330	29- to 35-week gestational-age infants	1453	• Randomised, double-blind, placebo-controlled trial • Arm 1: *Beyfortus* i.m. • Arm 2: placebo i.m.	• Primary endpoints: safety and efficacy	• FPCD: Q4 2016 • LPCD: Q4 2017 • Data readout: Q4 2018 • Primary endpoint met
Phase II/III MEDLEY NCT03959488	High-risk pre-term (born 35 weeks 0 day or less gestational-age) CHD and CLD infants eligible to receive Synagis	925	• Randomised, double-blind, palivizumab-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: *Synagis* i.m. • Global trial – 32 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, ADA and descriptive efficacy	• FPCD: Q3 2019 • LPCD: Q4 2020 • Data readout: Q2 2021 • Safety objective met
Phase II MUSIC NCT04484935	Immunocompromised children who are ≤24 months of age at the time of dose administration	100	• Open-label, uncontrolled, single-dose trial • *Beyfortus* i.m. • Route of administration: i.m. • Global trial – 8 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, ADA and efficacy	• FPCD: Q3 2020 • LPCD: Q1 2022 • Data readout: Q2 2023 • Primary endpoint met
Phase I NCT04840849	Healthy Chinese adults; age 18 to 45 years	24	• Randomised, double-blind, placebo-controlled • Arm 1: *Beyfortus* i.m. • Arm 2: placebo i.m. • China only	• Primary endpoint: PK parameters • Secondary endpoints: ADA and safety	• FPCD: Q2 2021 • LPCD: Q2 2021 • Data readout: Q2 2022

As of 28 July 2023.
Appendix: Glossary.

AZD3152 (SARS-CoV-2 LAAB)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SUPERNOVA** **NCT05648110**	Phase I: healthy adults; age 18 to 55 years Phase II: immuocompetent or immunoimpared adults 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) • Phase I (sentinel safety cohort): 56 healthy adults, age 18 to 55 years, randomised in a 5:2 ratio to receive AZD5156 or placebo • 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 • Phase II (sub-study, open-label): participants randomised 2:1 to receive 1200mg i.v. AZD3152 or 300mg i.m. *Evusheld* • 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 • 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 • Data anticipated: H2 2023
Phase I **LITTLE DIPPER** **NCT05872958**	Healthy adult participants; age 18 to 55 years	96	• Phase I, double-blind, placebo-controlled, multi-centre, dose exploration trial to evaluate the safety and PK of AZD3152 in healthy adult participants across different dose levels and routes of administration • Approximately 96 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 • Secondary endpoint: to evaluate ADA responses to AZD3152	• FPCD: Q2 2023 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

BioPharmaceuticals: early-stage development

AZD0186 (oral sm GLP-1Ra)

Type 2 diabetes

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05694741**	Healthy volunteers	24	• Randomised, sequential assignment, sponsor-open, placebo-controlled	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2022 • LPCD: Q2 2023 • Trial discontinued due to strategic portfolio prioritisation

As of 28 July 2023.
Appendix: Glossary.

AZD0780 (PCSK9 inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05384262**	Healthy adults	132	• Randomised, placebo-controlled SAD/MAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q2 2022 • Data anticipated: H2 2023

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Appendix: Glossary.

AZD2373

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04269031**	Healthy volunteers	30	• SAD dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD2373 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q1 2020 • LPCD: Q3 2021 • Data readout: Q3 2022
Phase I **NCT05351047**	Healthy volunteers	24	• MAD dose escalation in 3 cohorts with 6 volunteers per cohort receiving AZD2373 and 2 volunteers per cohort receiving placebo • Arm 1: AZD2373 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • 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 • LPCD: Q1 2023 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

AZD2693 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb FORTUNA NCT05809934	Non-cirrhotic non-alcoholic steatohepatitis (NASH) with fibrosis	232	• Randomised, double-blind, placebo-controlled, multi-centre trial • Arm 1: AZD2693 s.c. dose 1 • Arm 2: AZD2693 s.c. dose 2 • Arm 3: placebo s.c. • Global trial	• Primary endpoints: efficacy, safety and tolerability of AZD2693	• FPCD: Q2 2023 • Data anticipated: >2024
Phase I NCT04142424	Healthy volunteers	72	• SAD with 6 cohorts with 6 volunteers receiving AZD2693 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2019 • LPCD: Q3 2021 • Data readout: Q1 2022
Phase I NCT04483947	NASH/NAFLD F0-F3	74	• MAD with 4 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q2 2021 • Data anticipated: H1 2024
Phase I NCT05107336	Healthy volunteers	44	• MAD with 4 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • JP only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2021 • LPCD: Q4 2022 • Data anticipated: H2 2023

As of 28 July 2023.
Appendix: Glossary.

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase II **Re-PHiRE** **NCT05737940**	Heart failure and pulmonary hypertension due to left heart disease	220	• Randomised, double-blind, placebo-controlled, multi-centre, dose-ranging trial • Arm 1: AZD3427 (high dose) • Arm 2: AZD3427 (medium dose) • Arm 3: AZD3427 (low dose) • Arm 4: placebo • 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 • Data anticipated: >2024
Phase I **NCT04630067**	Healthy volunteers (SAD) Heart failure (MAD)	104	• Multi-centre SAD and MAD trial • Part A: SAD 6 cohorts • Arm 1: AZD3427 • Arm 2: placebo • Part B: MAD • Arm 1: AZD3427 • Arm 2: placebo • US only	• Primary endpoints: safety and tolerability	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data readout: Q4 2022

As of 28 July 2023.
Appendix: Glossary.

AZD5462 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04994106**	Healthy volunteers (SAD/MAD)	98	• Single-centre SAD and MAD • Part A: SAD (8 cohorts) • Arm 1: AZD5462 • Arm 2: placebo • Part B: MAD (5 cohorts) • Arm 1: AZD5462 • Arm 2: placebo • US only	• Primary endpoints: safety and tolerability	• FPCD: Q4 2021 • LPCD: Q3 2022 • Data readout: Q2 2023

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Appendix: Glossary.

AZD6234 (long-acting amylin)

Obesity with related comorbidities

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05511025**	Healthy participants who are overweight or obese	64	• SAD trial	• Primary endpoint: safety	• FPCD: Q4 2022 • Data anticipated: H2 2023

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Appendix: Glossary.

AZD7503 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05143905	Healthy volunteers	56	• SAD with 7 cohorts with 8 volunteers receiving AZD7503 and 2 volunteers receiving placebo in each cohort • Arm 1: AZD7503 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2021 • Data anticipated: H2 2023
Phase I NCT05560607	NAFLD or NASH	14	• Single-centre, open-label Phase I trial to assess knockdown of hepatic HSD17B13 mRNA PK, safety and tolerability following multiple doses of AZD7503	• Primary endpoint: safety and tolerability • Secondary endpoint: change in HSD17B13 mRNA Expression	• FPCD: Q3 2022 • Data anticipated: H2 2024
Phase I NCT05864391	NASH F1-F3	60	• Randomised, single-centre blind MAD trial	• Primary endpoint: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q3 2023 • Data anticipated: H2 2024

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Appendix: Glossary.

AZD9550 (GLP-1-glucagon agonist)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05848440**	Healthy volunteers	64	• SAD trial	• Primary endpoints: safety and tolerability	• FPCD: Q2 2023 • Data anticipated: H1 2024

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Appendix: Glossary.

balcinrenone/dapagliflozin (MR modulator + SGLT2 inhibitor)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **MIRACLE** **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 • Arm 1: AZD9977 A + *Farxiga* 10mg • Arm 2: AZD9977 B + *Farxiga* 10mg • Arm 3: AZD9977 C + *Farxiga* 10mg • Arm 4: *Farxiga* 10mg • 12 weeks • Global trial – 19 countries	• Primary endpoint: percent change from baseline in UACR at 12 weeks • Secondary endpoints: percent change from baseline in UACR at 12 weeks to assess dose-response relationship; dose-response relationship of *Farxiga* and 3 doses of AZD9977 combined with *Farxiga* on UACR; safety, tolerability and serum potassium values; eGFR	• FPCD: Q2 2021 • Data anticipated: H2 2023

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Appendix: Glossary.

MEDI6570

Cardiovascular

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT04610892**	Post-myocardial infarction	400	• Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure events • Arm 1: MEDI6570 (high dose) • Arm 2: MEDI6570 (medium dose) • Arm 3: MEDI6570 (low dose) • Arm 4: placebo • Global trial – US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Australia and Russia	• Primary endpoints: safety and efficacy	• FPCD: Q4 2020 • LPCD: Q4 2022 • Data anticipated: H1 2024

As of 28 July 2023.
Appendix: Glossary.

MEDI8367

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04365218**	Healthy volunteers CKD	12	• SAD trial • 6 cohorts • Arm 1: MEDI8367 s.c. • Arm 2: placebo s.c. • US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and ADA	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q2 2022

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Appendix: Glossary.

mitiperstat (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05236543	Healthy volunteers	14	• Open-label • mitiperstat vs. mitiperstat and itraconazole • UK only	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2022 • LPCD: Q3 2022 • Data readout: Q1 2023
Phase I NCT05457270	Healthy volunteers	30	• Open-label • 2-period, 2-treatment, single-dose, crossover trial • Period 1: single oral dose mitiperstat Formulation A or B on Day 1 • Period 2: single oral dose mitiperstat Formulation A or B on Day 1 • US only	• Primary endpoints: relative bioavailability and PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q3 2022 • LPCD: Q3 2022 • Data readout: Q1 2023

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Appendix: Glossary.

mitiperstat (MPO inhibitor)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II **COSMOS** **NCT05638737**	NASH	90	• Randomised, placebo-controlled, double-blind • Arm 1: 5mg mitiperstat • Arm 2: placebo • Global trial	• Primary endpoints: safety, tolerability and PD parameters	• FPCD: Q1 2023 • Data anticipated: H2 2024
Phase I **NCT05751759**	Participants with hepatic impairment and participants with normal hepatic function		• Phase I, single dose, non-randomised, open-label, parallel-group trial	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q1 2023 • Data anticipated: H2 2024

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Appendix: Glossary.

zibotentan (endothelin receptor antagonist)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **ZENITH-CKD** **NCT04724837**	CKD	495	• Arm 1: zibotentan dose A + *Farxiga* 10mg QD • Arm 2: zibotentan dose B + *Farxiga* 10mg QD • Arm 3: *Farxiga* 10mg + placebo QD • Global trial	• Primary endpoint: change in log-transformed UACR from baseline to Week 12 zibotentan dose B/*Farxiga* 10mg vs. *Farxiga* 10mg • Secondary endpoints: change in log-transformed UACR from baseline to Week 12 zibotentan dose A/*Farxiga* 10mg vs. *Farxiga* 10mg; change in blood pressure, least squares mean change of UACR, change in eGFR at predetermined timepoints and number of participants experiencing adverse events	• FPCD: Q2 2021 • Data anticipated: H2 2023

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Appendix: Glossary.

atuliflapon (FLAP inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **FLASH** **NCT05251259**	Patients with moderate-to-severe uncontrolled asthma	1102	• Randomised, placebo-controlled, double-blind, multi-centre, 2-part trial with a lead-in PK cohort • PK cohort • Arm 1: atuliflapon • Arm 2: placebo • Part 1 • Arm 1: atuliflapon • Arm 2: placebo • Part 2 • Arm 1: atuliflapon dose A • Arm 2: atuliflapon dose B • Arm 3: atuliflapon dose C • Arm 4: placebo • Global trial	• Primary endpoint: time to first CompEx asthma event	• FPCD: Q2 2022 • Data anticipated: >2024

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Appendix: Glossary.

AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04769869**	Healthy volunteers and patients with mild asthma	137	• SAD/MAD/POM trial • Part 1 SAD • Arm 1: AZD4604 (DPI) • Arm 2: placebo (DPI) • Part 2 MAD • Arm 1: AZD4604 (DPI) • Arm 2: placebo (DPI) • Part 3 POM • Arm 1: AZD4604 (DPI) • Arm 2: placebo (DPI) • UK only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and FENO	• FPCD: Q4 2021 • Data anticipated: H2 2023

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Appendix: Glossary.

AZD5055 (oral porcupine inhibitor)

Idiopathic pulmonary fibrosis (IPF) and other ILDs with progressive fibrosis

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05134727**	Healthy volunteers	90	• SAD/MAD trial • Part 1: SAD • Arm 1: AZD5055 (oral suspension) • Arm 2: placebo (oral suspension) • Part 2: MAD • Arm 1: AZD5055 (oral suspension) • Arm 2: placebo (oral suspension)	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters	• FPCD: Q4 2021 • LPCD: Q2 2023 • Data readout: Q2 2023
Phase I **NCT05630677**	Healthy volunteers	18	• BA trial to compare film-coated tablet with oral suspension and to assess the effect of food and an acid reducing agent on PK of AZD5055 in healthy volunteers	• Primary endpoints: bioavailability and PK parameters	• FPCD: Q4 2022 • LPCD: Q1 2023 • Data readout: Q2 2023

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Appendix: Glossary.

AZD6793 (IRAK4)

Inflammatory diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05662033	Healthy volunteers	133	• Single-blind, randomised, placebo-controlled trial to investigate the safety, tolerability and PK of oral AZD6793 following single and multiple ascending doses in healthy subjects	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2022 • Data anticipated: H1 2024

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Appendix: Glossary.

AZD7798 (humanised mAb)

Crohn's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05452304**	Healthy volunteers	64	• SAD • Arm1: AZD7798 • Arm2: placebo	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and immunogenicity	• FPCD: Q3 2022 • Data anticipated: H2 2023

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Appendix: Glossary.

AZD8630 (inhaled TSLP)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05110976 Partnered (AMGEN)	Healthy volunteers and patients with asthma	232	• SAD and MAD trial	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters and FENO	• FPCD: Q1 2022 • Data anticipated: H2 2023

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Appendix: Glossary.

elarekibep (AZD1402, IL-4 receptor alpha antagonist)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **APATURA** **NCT04643158**	Patients with asthma on medium dose inhaled corticosteroids	225	• Randomised, placebo-controlled, double-blinded, multi-centre, 2-part trial • Part 1: population with asthma controlled on medium dose ICS-LABA • Part 1a • Arm 1: AZD1402 dose 1 (low) (DPI) • Arm 2: AZD1402 dose 2 (DPI) • Arm 3: placebo • Part 1b • Arm 1: AZD1402 dose 3 (high) (DPI) • Arm 2: placebo • Part 2: population uncontrolled on medium dose ICS-LABA • Arm 1: AZD1402 dose 1 (DPI) • Arm 2: AZD1402 dose 2 (DPI) • Arm 3: placebo • Global trial – Ukraine, Australia, Germany, Hungary, Korea, Poland, Spain, UK and Taiwan	• Primary endpoints (Part 1): safety and tolerability, PK parameters • Primary endpoint (Part 2): change in FEV1	• FPCD: Q2 2021 • Trial discontinued due to safety/efficacy

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Appendix: Glossary.

mitiperstat (MPO inhibitor)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase II **CRESCENDO** **NCT05492877**	Moderate to severe COPD; age 40 to 80	288	• Randomised, double-blind trial • Arm 1: 5mg mitiperstat • Arm 2: placebo • Global trial – 14 countries	• Primary endpoint: time to first COPD CompEx event • 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 • Data anticipated: H1 2024

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Appendix: Glossary.

AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05209334 Partnered (National Institute on Drug Abuse)	Healthy volunteers	36	• Randomised, double-blind MAD trial • Arm 1: AZD4041 • Arm 2: placebo • Canada only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q1 2022 • LPCD: Q2 2022 • Data readout: Q4 2022 • Primary end point met
Phase I NCT05587998 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 • LPCD: Q2 2023 • Data anticipated: H2 2023

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Appendix: Glossary.

MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain, migraine prevention

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04198558**	Healthy volunteers	64	• SAD trial • Arm 1: MEDI0618 i.v. • Arm 2: placebo i.v. • Arm 3: MEDI0618 s.c. • Arm 4: placebo s.c. • Europe only	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2019 • LPCD: Q1 2022 • Data readout: Q2 2022
Phase I **NCT05714254**	Healthy volunteers	48	• Randomised, double-blind, placebo-controlled MAD trial • Arm 1: MEDI0618 i.v. or placebo • Arm 2: MEDI0618 s.c. or placebo	• Primary endpoints: safety, tolerability and PK parameters	• FPCD: Q4 2022 • Data anticipated: H1 2024

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Appendix: Glossary.

MEDI1341 (alpha-synuclein mAb)

Multiple system atrophy

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT05526391** **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 • Early PK cohort • Arm 1: TAK-341/MEDI1341 i.v. • Arm 2: placebo i.v. • Main cohort • Arm 3: TAK-341/MEDI1341 i.v. • Arm 4: placebo i.v.	• Primary endpoints: efficacy, change from baseline on modified Unified Multiple System Atrophy Rating Scale at 52 weeks • Secondary endpoints: PK parameters, safety and efficacy	• FPCD: Q4 2022 • Data anticipated: >2024

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Appendix: Glossary.

MEDI1341 (alpha-synuclein mAb)

Parkinson’s disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04449484** **Partnered (Takeda)**	Parkinson's disease	25	• MAD trial • Arm 1: MEDI1341 i.v. • Arm 2: placebo i.v. • US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK and PD parameters	• FPCD: Q3 2020 • LPCD: Q3 2021 • Data readout: Q4 2022

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Appendix: Glossary.

MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04675034	Painful osteoarthritis of the knee	350	• MAD trial • Arm 1: MEDI7352 s.c. • Arm 2: placebo s.c. • Global – 7 countries	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK and PD parameters, ADA	• FPCD: Q1 2021 • LPCD: Q3 2022 • Data anticipated: H2 2023
Phase IIa NCT03755934	Painful diabetic neuropathy	271	• MAD trial • Arm 1: MEDI7352 i.v. • Arm 2: placebo i.v. • Europe only	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK and PD parameters	• FPCD: Q4 2018 • Data anticipated: H2 2024

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Appendix: Glossary.

Rare Disease: approved medicines and late-stage pipeline

Koselugo (selumetinib, MEK inhibitor)

Neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase III **KOMET** **NCT04924608**	Adult age ≥18 years with NF1 who have symptomatic, inoperable PN Available baseline chronic target PN pain score	146	• Multi-centre, international trial with a parallel, randomised, double-blind, placebo-controlled, 2 arm design • Arm 1: *Koselugo* 25mg/m2 BID • Arm 2: placebo BID until end of Cycle 12, then cross-over to *Koselugo* 25mg/m2 BID	• Primary endpoint: ORR by end of Cycle 16 on *Koselugo* vs. placebo as determined by ICR per REiNS criteria • Secondary endpoint: change in baseline of chronic PN-pain intensity on *Koselugo* vs. placebo	• FPCD: Q4 2021 • Data anticipated: H2 2024
Phase I/II **SPRINKLE** **NCT05309668**	Paediatric (age 1 to 6 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*	• Primary endpoints: selumetinib AUC0-12 derived after single dose administration [time frame: pre-dose and 1, 2, 3, 4, 6, 8 and 10-12 hours after selumetinib 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 • Data anticipated: H1 2024
Phase I **China PK/Safety/Efficacy** **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 • Secondary endpoint: efficacy (ORR, DoR; TTR; PFS)	• FPCD: Q1 2021 • Data anticipated: H2 2023
Phase I **Food Effect/GI Tolerability** **NCT05101148**	Adolescents aged ≥12 to <18 years at trial entry with a clinical diagnosis of NF1-related PN *Koselugo* with a low-fat meal compared to fasted state	24	• Single-arm, multiple dose, sequential, two or three period trial • *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 • Data anticipated: H2 2023

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Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Haematology, nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1210-TM-313** **NCT04543591**	Thrombotic microangiopathy-associated haematopoietic stem cell transplant	184	• Arm 1: *Ultomiris* Q8W • Arm 2: placebo	• Primary endpoint: TMA response • Secondary endpoints: time to TMA response, TMA relapse	• FPCD: Q4 2020 • Data anticipated: >2024
Phase III **ALXN1210-TM-314** **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 • Secondary endpoints: time to TMA response, proportion of participants with TMA relapse	• FPCD: Q4 2020 • Data anticipated: >2024
Phase III **ARTEMIS (ALXN1210-CSA-AKI-318)** **NCT05746559**	CSA-AKI	736	• Randomised, double-blind, placebo-controlled, muticentre trial • *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 • Data anticipated: >2024
Phase II **SANCTUARY (ALXN1210-NEPH-202)** **NCT04564339**	Proliferative lupus nephritis or immunoglobulin A nephropathy	120	• Arm 1: LN cohort, *Ultomiris* • Arm 2: LN cohort, placebo • Arm 3: IgAN cohort, *Ultomiris* • Arm 4: IgAN cohort, placebo	• Primary endpoint: percentage change in proteinuria from baseline to Week 26 • Secondary endpoints: percentage change in proteinuria from baseline to Week 50	• FPCD: Q1 2021 • Data anticipated: H2 2024

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Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1210-NMO-307** **NCT04201262**	Neuromyelitis optica spectrum disorder	58	• Arm 1: *Ultomiris* Q8W	• Primary endpoint: time to first adjudicated on-trial relapse	• FPCD: Q4 2019 • LPCD: Q1 2021 • Data readout: Q2 2022 • Primary endpoint met
Phase II/III **ALXN1210-DM-310** **NCT04999020**	Dermatomyositis	150	• Arm 1: *Ultomiris* • Arm 2: placebo	• Primary endpoint: improvement response on IMACS-TIS	• FPCD: Q4 2021 • Data anticipated: H1 2024
Phase II/III **ALXN1210-NMO-317** **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 • Data anticipated: >2024

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Appendix: Glossary.

acoramidis (ALXN2060)

ATTR-CM

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2060-TAC-302** **NCT04622046**	ATTR-CM	22	• Arm 1: 800mg acoramidis administered twice daily	• 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 • Data anticipated: H1 2024

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Appendix: Glossary.

anselamimab (CAEL-101, fibril-reactive mAb)

AL amyloidosis

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAEL101-302 NCT04512235	Mayo Stage IIIa amyloidosis	267	• Arm 1: anselamimab combined with SoC for PCD • Arm 2: placebo combined with SoC for PCD	• Primary endpoint: time from first dose of trial drug until death or end of trial • Secondary endpoint: change in distance walked during a six-minute walk test and quality of life measures	• FPCD: Q4 2020 • Data anticipated: >2024
Phase III CAEL101-301 NCT04504825	Mayo Stage IIIb amyloidosis	124	• Arm 1: anselamimab combined with SoC for PCD • Arm 2: placebo combined with SoC for PCD	• Primary endpoint: time from first dose of trial drug until death or end of trial • Secondary endpoint: change in distance walked during a six-minute walk test and quality of life measures	• FPCD: Q1 2021 • Data anticipated: H2 2024
Phase II CAEL101-203 NCT04304144	Mayo Stage I, Stage II and Stage IIIa amyloidosis	25	• Arm 1: anselamimab combined with SoC CyBorD • Arm 2: placebo combined with SoC CyBorD and daratumumab	• Primary endpoint: occurrence of DLT during the first 4 weeks of therapy • Secondary endpoint: AUC (plasma curve concentration)	• FPCD: Q1 2020 • Data anticipated: H1 2024

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Appendix: Glossary.

danicopan (ALXN2040, factor D inhibitor)

Haematology, ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2040-PNH-301** **NCT04469465**	PNH with clinically significant EVH	84	• Arm 1: danicopan + C5 Inhibitor • Arm 2: placebo + C5 Inhibitor	• Primary endpoint: change from baseline in haemoglobin at Week 12 • Secondary endpoint: percentage of participants with transfusion avoidance	• FPCD: Q1 2021 • Data readout: Q3 2022 • Primary endpoint met
Phase III **ALXN2040-PNH-303** **NCT05389449**	PNH	100	• Arm 1: danicopan together with background C5 inhibitor therapy	• Primary endpoint: participants experiencing TEAEs and serious TEAEs	• FPCD: Q4 2022 • Data anticipated: >2024
Phase II **ALXN2040-GA-201** **NCT05019521**	Geographic atrophy	332	• Arms 1-3: danicopan dosed at 100mg-400mg QD • Arm 4: placebo	• Primary endpoint: mean rate of change from baseline at Week 52 in the square root of total GA lesion area in the trial eye as measured by FAF	• FPCD: Q3 2021 • Data anticipated: >2024

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Appendix: Glossary.

gefurulimab (ALXN1720, anti-C5 bispecific mini-body)

Neurology, nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1720-MG-301** **NCT05556096**	Generalised myasthenia gravis	200	• 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 • Arm 2: placebo	• Primary endpoint: change from baseline in MG-ADL total score at Week 26	• FPCD: Q4 2022 • Data anticipated: >2024
Phase I **ALXN1720-NEPH-102** **NCT05314231**	Proteinuria	12	• 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 • Data anticipated: H2 2023

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Appendix: Glossary.

Rare Disease: early-stage development

ALXN1850 (next-generation asfotase alfa)

Hypophosphatasia

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1850-HPP-101** **NCT04980248**	Hypophosphatasia	15	• Arm 1: ALXN1850, 3 cohorts at low, medium and high dosages	• Primary endpoint: incidence of TEAEs and TESAEs	• FPCD: Q3 2021 • Data readout: Q4 2022 • Primary endpoint met

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Appendix: Glossary.

ALXN1910 (next-generation TNSALP ERT)

Bone metabolism

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1910-HV-101** **NCT05307978**	Healthy adults	48	• Randomised, placebo-controlled SAD	• Primary endpoint: safety	• FPCD: Q2 2022 • Data readout: Q2 2023

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Appendix: Glossary.

ALXN1920 (kidney-targeted factor H fusion protein)

Nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1920-HV-101** **NCT05751642**	Healthy adults	48	• Randomised, double-blind, placebo-controlled, SAD	• Primary endpoint: safety and tolerability • Secondary endpoints: PK/PD parameters	• FPCD: Q2 2023 • Data anticipated: H1 2024

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Appendix: Glossary.

ALXN2030 (siRNA targeting complement C3)

Nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN2030-HV-101** **NCT05501717**	Healthy volunteers	48	• Randomised, placebo-controlled SAD	• Primary endpoint: safety	• FPCD: Q4 2022 • Data anticipated: H2 2024

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Appendix: Glossary.

ALXN2080 (oral factor D inhibitor)

Complement-mediated disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN2080-HV-101** **NCT05428696**	Healthy volunteers	100	• SAD/MAD trial	• Primary endpoints: safety and tolerability, PK and PD parameters	• FPCD: Q3 2022 • Data anticipated: H2 2023

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Appendix: Glossary.

tarperprumig (ALXN1820, anti-properdin)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **PHOENIX (ALXN1820-SCD-201)** **NCT05565092**	SCD	30	• Randomised, open-label • Arm 1: 300mg tarperprumig QW • Arm 2: 600mg tarperprumig Q4W • Arm 3: 300mg tarperprumig Q8W (optional cohort)	• Primary endpoints: TEAEs and SAEs • Secondary endpoints: PK parameters	• FPCD: Q2 2023 • Data anticipated: H2 2024
Phase I **ALXN1820-HV-101** **NCT04631562**	Healthy volunteers	60	• Arm 1: tarperprumig administered s.c. or i.v., multiple ascending doses • Arm 2: placebo	• Primary endpoint: participants with TEAEs	• FPCD: Q1 2021 • Data readout: Q1 2023

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vemircopan (ALXN2050, factor D inhibitor)

Haematology, nephrology, neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ACH228-110** **NCT04170023**	PNH	28	• Arm 1: vemircopan monotherapy with groups including treatment-naïve, C5 inhibitor treatment experienced and patients previously receiving danicopan	• Primary endpoint: change in haemoglobin relative to baseline • Secondary endpoints: number of participants who have transfusion avoidance and change in LDH relative to baseline	• FPCD: Q4 2019 • Data anticipated: >2024
Phase II **ALXN2050-gMG-201** **NCT05218096**	Generalised myasthenia gravis	70	• Arm 1: vemircopan 180mg • Arm 2: vemircopan 120mg • Arm 3: placebo followed by vemircopan	• Primary endpoint: MG-ADL total score reduction of ≥2 points in any 4 consecutive weeks during the first 8 weeks and who did not receive rescue therapy	• FPCD: Q2 2022 • Data anticipated: >2024
Phase II **ALXN2050-NEPH-201** **NCT05097989**	Lupus nephritis or immunoglobulin A nephropathy	126	• Arm 1 – LN cohort: vemircopan 180mg • Arm 2 – LN cohort: vemircopan 120mg • Arm 3 – LN cohort: placebo • Arm 4 – IgAN cohort: vemircopan 180mg • Arm 5 – IgAN cohort: vemircopan 120mg • Arm 6 – IgAN cohort: placebo	• Primary endpoint (both cohorts): percentage change in proteinuria from baseline to Week 26	• FPCD: Q3 2022 • Data anticipated: >2024
Phase I **ALXN2050-HV-109** **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 • Arm 2: moderate IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 • Arm 3: severe IHF, 120mg vemircopan BID orally on Days 1 through 3, 120mg orally on the morning of Day 4 • 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 • Primary endpoint (Arm 2): AUCt of plasma vemircopan after steady-state • Primary endpoint (Arm 3): Cmax,ss of vemircopan • Primary endpoint (Arm 4): Tmax,ss following vemircopan	• FPCD: Q2 2022 • Data anticipated: H1 2024

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Appendix: Glossary.

List of abbreviations

14C	Radioactive isotope of carbon, Carbon 14
1L, 2L, 3L	1st, 2nd or 3rd line
5-FU	5-fluorouracil
A2AR	Adenosine A2A receptor
ACQ	Asthma control questionnaire
ACR	American college of rheumatology response scoring system
ADA	Anti-drug antibodies
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
AE	Adverse event
aHUS	Atypical haemolytic uraemic syndrome
AI	Auto-injector
AKT	Protein kinase B
ALK	Anaplastic large-cell lymphoma kinase
ALSFRS-R	Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised
APFS	Accessorised pre-filled syringe
AQLQ	Asthma quality of life questionnaire
AS	Albuterol sulphate
ATM	Ataxia-telangiectasia mutated kinase
ATR	Ataxia telangiectasia and rad3-related protein
ATTR-CM	Transthyretin amyloid cardiomyopathy
AUC	Area under curve
AUEC	Area under the effect-time curve
B7RP	B7-related protein-1
BA	Bioavailability
BAFF	B-cell activating factor
BCG	Bacillus Calmette–Guérin
BCMA	B-cell maturation antigen
BDA	Budesonide albuterol
BFF	Budesonide and formoterol fumarate
BGF	Budesonide, glycopyrronium and formoterol fumarate
BICR	Blinded independent central review
BID	Bis in die (twice per day)
BIG	Big ten cancer research consortium
BMD	Bone mineral density
BMFI	Bone metastasis-free interval
BMI	Body mass index
BRCAwt	Breast cancer wild-type gene
BRD4	Bromodomain-containing protein 4
BTC	Biliary tract carcinoma
BTK	Bruton's tyrosine kinase
CA-125	Cancer antigen 125
CAD	Coronary artery disease
CBR	Clinical benefit rate
CD	Cluster of differentiation
CDK	Cyclin-dependent kinase
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
CKD	Chronic kidney disease
CLL	Chronic lymphocytic leukaemia
CMAX	Maximum observed plasma concentration
C-MET	Tyrosine-protein kinase Met
CNS	Central nervous system
COPD	Chronic obstructive pulmonary disease
CR	Complete response
CRC	Colorectal cancer
CrCl	Creatinine clearance
CRR	Complete response rate
CTC	Circulating tumour cell
CTLA-4	Cytotoxic T-lymphocyte–associated antigen 4
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CVRM	Cardiovascular renal and metabolism
CXCR2	C-X-C Motif chemokine receptor 2
DB	Double blind
DC	Disease control
DCR	Disease control rate
DDI	Drug-drug Interaction
dECG	Differentiated electrocardiogram
DFS	Disease free survival
DLBCL	Diffuse large B-cell lymphoma
DLT	Dose-limiting toxicity
DMARDs	Disease-modifying antirheumatic drugs
DNA	Deoxyribonucleic acid
DoCR	Durability of complete response
dNCC	Directly Measured Non-ceruloplasmin-bound Copper
DoR	Duration of response
DPI	Dry powder inhaler
DRFI	Disease recurrence-free interval
DXA	Dual energy X-ray absorptiometry
EBRT	External beam radiation therapy
ECG	Electrocardiogram
EFS	Event-free survival
eGFR	Estimated glomerular filtration rate
EGFR	Epidermal growth factor receptor
ER	Oestrogen receptor
ERK	Extracellular signal-regulated kinase
ESCC	Esophageal squamous cell carcinoma
ESR	Externally sponsored trial
ESR1	Oestrogen receptor 1
ET	Endocrine therapy
FAF	Fundus Autofluorescence
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FGFR	Fibroblast growth factor receptor
FLAP	5-lipoxygenase-activating protein
FPDC	First patient commenced dosing
FPG	Fasting plasma glucose
GA	Gestational age
GA	Geographic atrophy
GBM	Glioblastoma
gBRCAm or tBRCAm	Germline or tumour (somatic) BRCA mutation
GEJ	Gastric/gastro-oesophogeal junction
GFF	Glycopyrronium and formoterol fumarate
GLP-1	Glucagon-like peptide-1
GMFRs	Geometric mean fold rises
GMTs	Geometric mean titers
hADME	Human mass balance
HAI	Haemagglutination-inhibition
HbA1c	Haemoglobin A1c
HCC	Hepatocellular carcinoma
HD	High dose
HDL-C	High-density lipoprotein cholesterol
HER2	Human epidermal growth factor receptor 2
HF	Heart failure
HFpEF	Heart failure with preserved ejection fraction

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Appendix: Glossary.

List of abbreviations

HFrEF	Heart failure with reduced ejection fraction
HGFR	Met/hepatocyte growth factor receptor
HGSC	High grade serous carcinoma
hHF	Hospitalisation for heart failure
HIF-PHI	Hypoxia inducible factor - prolyl hydroxylase inhibitor
HNSCC	Head and neck squamous-cell carcinoma
HPV	Human papillomavirus
HRD	Homologous recombination deficiency
HRRm	Homologous recombination repair mutation
i	inhibitor
IA	Investigator-assessed
ICS	Inhaled corticosteroid
ICU	Intensive care unit
IDFS	Invasive disease-free survival
IgAN	Immunoglobulin A nephropathy
IL	Interleukin
i.m.	Intramuscular
IRC	Independent review committee
ISS	Investigator-sponsored studies
i.v.	Intravenous
J-SD	Japanese single dose
Ki67	Protein that is encoded by the MKI67 gene in human
LAAB	Long-acting antibody
LABA	Long -acting beta agonist
LAMA	Long-acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LCM	Lifecycle management
LDH	Lactate dehydrogenase
LN	Lupus nephritis
LOCS III	Lens opacities classification system III
LPCD	Last patient commenced dosing
LV	Left ventricle
m	Mutation
mAb	Monoclonal antibody
MABA	Muscarinic antagonist-beta2 agonist
MACE	Major adverse cardiac events
MAD	Multiple ascending dose
MCC	Mucociliary clearance
MCL	Mantle cell lymphoma
MCL1	Myeloid leukemia cell differentiation protein 1
mCRPC	Metastatic castrate-resistant prostate cancer
MD	Medium dose
MDI	Metered-dose inhaler
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Tyrosine-protein kinase Met
MG-ADL	Myasthenia Gravis-Activities Of Daily Living
MI	Myocardial infarction
MMT	Mixed meal test
MPO	Myeloperoxidase
mPR	Major pathological response
MRI	Magnetic resonance imaging
MTD	Maximum tolerated dose
NaC	Sodium channel
NCI	National cancer institute (US)
NCPV	Noncalcified plaque volume
NF1	Neurofibromatosis type 1
NGF	Nerve growth factor
NHL	Non-Hodgkin's lymphoma
NIH	National Institute of Health (US)
NKG2a	Natural killer cell C-type lectin receptor G2A
NME	New molecular entity
NRG	National clinical trials network in oncology (US)
NSCLC	Non-small cell lung cancer
OCS	Oral corticosteroid
OD	Once daily
OGTT	Oral glucose tolerance test
OR	Objective response
ORR	Objective response rate
OS	Overall survival
PARP	Poly ADP ribose polymerase
PASI	Psoriasis area severity index
PBD	Pyrrolobenzodiazepine
pCR	Pathological complete response
PD	Pharmacodynamics
PD-1	Programmed cell death protein 1
PDAC	Pancreatic ductal adenocarcinoma
PDE4	Phosphodiesterase type 4
PD-L1	Programmed death-ligand 1
PET	Positron-emission tomography
PFS	Progression free survival
PgR	Progesterone receptor
PI3K	Phosphoinositide 3-kinase
PIK3CA	Phosphatidylinositol 3 kinase catalytic alpha gene
PK	Pharmacokinetics
PLL	Prolymphocytic leukaemia
pMDI	Pressurised metered dose inhaler
PN	Plexiform neurofibromas
PNH	Paroxysmal nocturnal haemoglobinuria
POC	Proof of concept
POM	Proof of mechanism
pPCI	Primary percutaneous coronary intervention
PR	Partial response
pre-BD	Pre-bronchodilator
PRO	Patient reported outcome
PRR	Recurrent platinum resistant
PS	Propensity score
PSA	Prostate-specific antigen
PSC	Pulmonary sarcomatoid carcinoma
PSMA	Prostate-specific membrane antigen
PTEN	Phosphatase and tensin homolog gene
Q2,3,4,8W	Quaque (every) two, three... weeks
QD	Quaque in die (once a day)
QID	Quarter in die (four times a day)
QOD	Quaque altera die (every other day)
QoL	Quality of Life
QTcF	Corrected QT interval by Fredericia
RA	Rheumatoid Arthritis
RAAS	Renin–angiotensin–aldosterone system
RECIST	Response evaluation criteria in solid tumours
RFS	Relapse-free survival
rhLCAT	Recombinant human Lecithin-cholesterol acyltransferase
RORγ	Related orphan receptor gamma
r/r	Relapsed/refractory
RSV	Respiratory syncytial virus
RT	Radiation therapy
R&I	Respiratory and Immunology
SABA	Short-acting beta2-agonist
SAD	Single ascending dose
SAE	Serious adverse event

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List of abbreviations

SBRT	Stereotactic body radiation therapy
s.c.	Subcutaneous
SCCHN	Squamous-cell carcinoma of the head and neck
SCLC	Small cell lung cancer
SD	Stable disease
SERD	Selective oestrogen receptor degrader
SGLT2	Sodium-glucose transport protein 2
SGRM	Selective glucocorticoid receptor modulator
SGRQ	Saint George respiratory questionnaire
SJC	Swollen joint count
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
STAT3	Signal transducer and activator of transcription 3
sUA	Serum uric acid
T2DM	Type 2 Diabetes Mellitus
T790M	Threonine 790 substitution with methionine
TACE	Transarterial Chemoembolization
TEAEs	Treatment-emergent adverse events
TESAEs	Treatment-emergent serious adverse events
TID	Ter in die (three times a day)
TJC	Tender joint count
TKI	Tyrosine kinase Inhibitor
TLR	Toll-like receptor 9
TMA	Thrombotic microangiopathy
TNBC	Triple negative breast cancer
TNF	Tumour necrosis factor
TSLP	Thymic stromal lymphopoietin
TTF	Time to treatment failure
TTNT	Time to next therapy
TTP	Time to tumour progression
UACR	Urine albumin creatinine ratio
UMEC	Umeclidinium
URAT1	Uric Acid Transporter 1
UWDRS	Unified Wilson Disease Rating Scale
VEGF	Vascular endothelial growth factor
V&I	Vaccine & Immune therapies
YTE	Triple-amino-acid (M252Y/S254T/T256E [YTE]) substitution

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Appendix: Glossary.