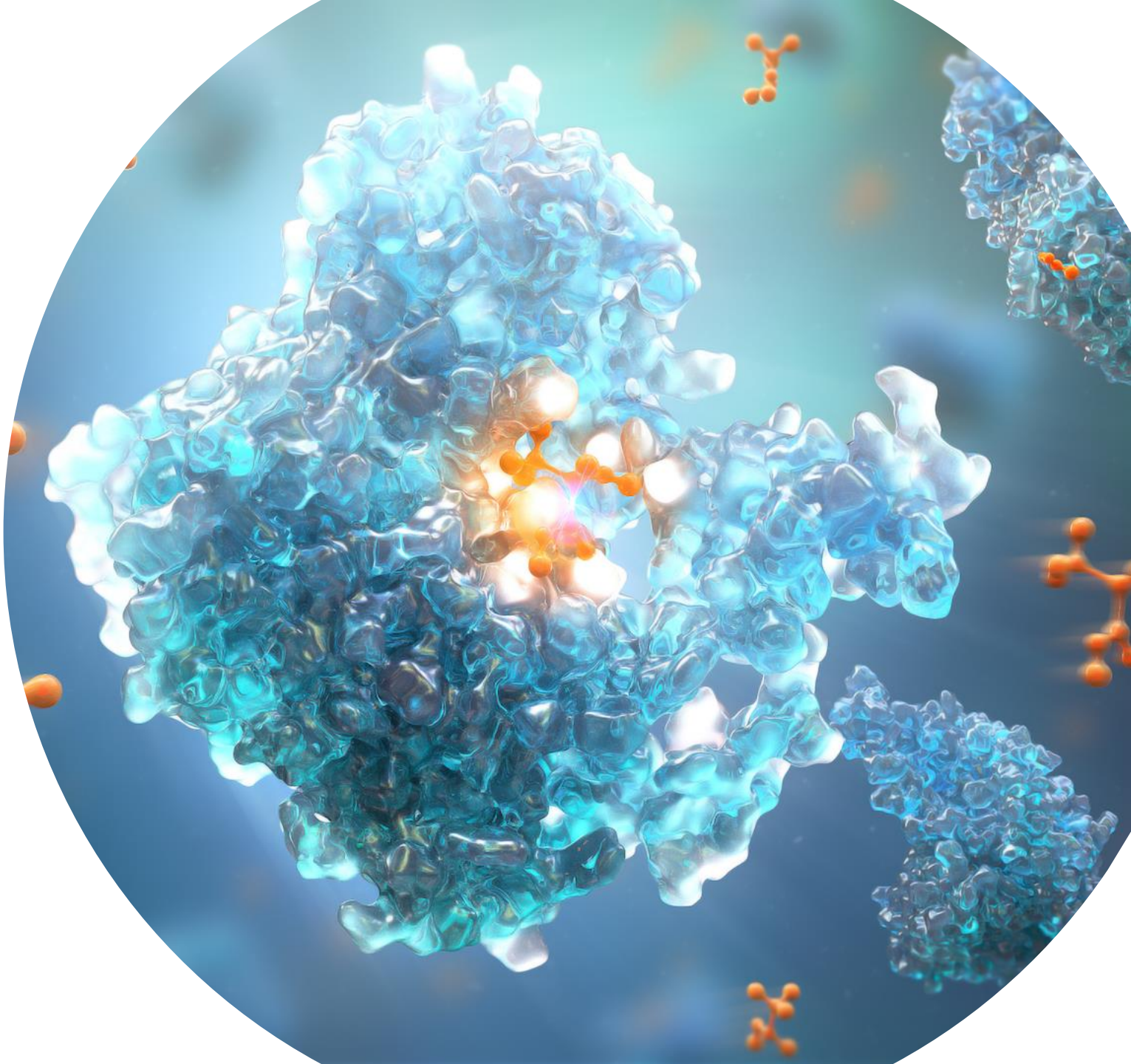
AstraZeneca

Clinical Trials Appendix

Q1 2023 Results Update



Upcoming pipeline catalysts: 2023 and 2024

Oncology BioPharmaceuticals Rare Disease

	H1 2023	H2 2023	2024	
Regulatory decision[1]	***Lynparza*** – prostate cancer (1L) (PROpel) (US) ***Farxiga*** – HFpEF (DELIVER) (US) ***Beyfortus*** – RSV (MELODY/MEDLEY) (US) ***Ultomiris*** – NMOSD (CHAMPION-NMOSD)	***Lynparza*** – prostate cancer (1L) (PROpel) (JP) ***Enhertu*** – HER2m NSCLC (2L+) (DESTINY-Lung01) (EU, JP) ***Enhertu*** – HER2-low breast cancer (3L) (DESTINY-Breast04) (CN) ***Calquence*** – CLL (ASCEND) (CN) ***Forxiga*** – HFpEF (DELIVER) (CN) **eplontersen** – hATTR-PN (NEURO-TTRansform) (US) ***Soliris*** – gMG (CN) ***Soliris*** – NMOSD (CN) ***Koselugo*** – NF1-PN (SPRINT) (CN)	***Imfinzi*** – biliary tract cancer (TOPAZ-1) (CN)	
Regulatory submission and/or acceptance	***Lynparza*** – gBRCA breast cancer (adjuvant) (OlympiA) (CN) **capivasertib** – HR+/HER2- breast cancer (1L) (CAPItello-291) ***Beyfortus*** – respiratory syncytial virus (CN) **danicopan** – PNH with extravascular haemolysis (US)	***Tagrisso*** – EGFRm NSCLC (1L) (FLAURA2) ***Tagrisso*** – EGFRm NSCLC (unresectable Stg. III) (LAURA) ***Imfinzi*** – NSCLC (neoadjuvant) (AEGEAN) ***Imfinzi*** – bladder cancer (muscle invasive) (NIAGARA) ***Imfinzi*** – bladder cancer (1L) (NILE) ***Enhertu*** – HER2+/HER2low gastric 3L (DESTINY-Gastric01) (CN) **Dato-DXd** – NSCLC (3L) (TROPION-Lung01) **Dato-DXd** – HR+/HER2- breast cancer (inoperable and/or met.) (TROPION-Breast01) ***Evusheld*** – COVID-19 (TACKLE/PROVENT) (CN) **AZD3152** – prevention of COVID-19 (SUPERNOVA) **danicopan** – PNH with extravascular haemolysis (JP)	***Tagrisso*** – EGFRm NSCLC (resectable, Stg. II/III) (NeoADAURA) ***Imfinzi + Imjudo*** – hepatocellular carcinoma (1L) (HIMALAYA) (CN) ***Imfinzi*** – NSCLC (unresectable, Stg. III) (PACIFIC-2) ***Imfinzi*** – liver cancer (locoregional) (EMERALD-1) ***Imfinzi*** – SCLC (limited-stage) (ADRIATIC) ***Lynparza*** – ovarian cancer (1L) (DUO-O) ***Lynparza*** – endometrial cancer (1L) (DUO-E) ***Lynparza*** – prostate cancer (1L) (PROpel) (CN) ***Enhertu*** – HER2-low breast cancer (2L) (DESTINY-Breast06) ***Enhertu*** – High-risk HER2+ early breast cancer (non-met.) (DESTINY-Breast11)	**capivasertib** – TNBC (locally adv./met.) (CAPItello-290) ***Calquence*** – MCL (1L) (ECHO) **roxadustat** – anaemia of myelodysplastic syndrome **tozorakimab** – acute respiratory failure (TILIA) ***Fasenra*** – bullous pemphigoid (FJORD) ***Fasenra*** – EGPA (MANDARA) ***Fasenra*** – HES (NATRON) **acoramidis** – ATTR-CM (ALXN2060-TAC-302) **anselamimab** – AL amyloidosis (CAEL101-302)
Key Phase III data readouts	***Tagrisso*** – EGFRm NSCLC (1L) (FLAURA2) **Dato-DXd** – NSCLC (3L) (TROPION-Lung01) **roxadustat** – anaemia of myelodysplastic syndrome	***Tagrisso*** – EGFRm NSCLC (unresectable Stg. III) (LAURA) ***Imfinzi*** – NSCLC (unresectable, Stg. III) (PACIFIC-2) ***Imfinzi*** – SCLC (limited-stage) (ADRIATIC) ***Imfinzi*** – liver cancer (locoregional) (EMERALD-1) ***Imfinzi*** – bladder cancer (muscle invasive) (NIAGARA) ***Imfinzi*** – bladder cancer (1L) (NILE) ***Lynparza*** – endometrial cancer (1L) (DUO-E) ***Enhertu*** – HER2-low breast cancer (2L) (DESTINY-Breast06) **capivasertib** – TNBC (locally adv./met.) (CAPItello-290) **Dato-DXd** – HR+/HER2- breast cancer (inoperable and/or met.) (TROPION-Breast01) ***Farxiga*** – myocardial infarction (DAPA-MI) ***Fasenra*** – EGPA (MANDARA) ***Fasenra*** – HES (NATRON) **AZD3152** – prevention of COVID-19 (SUPERNOVA)	***Tagrisso*** – EGFRm NSCLC (resectable, Stg. II/III) (NeoADAURA) ***Imfinzi*** – liver cancer (adjuvant) (EMERALD-2) ***Lynparza*** – PARP 1L BRCAwt ovarian cancer (MONO-OLA1) ***Enhertu*** – high-risk HER2+ early breast cancer (non-met.) (DESTINY-Breast11) ***Enhertu*** – HER2+ gastric cancer (2L) (DESTINY-Gastric04) ***Calquence*** – MCL (1L) (ECHO) ***Orpathys*** – NSCLC with MET exon 14 mutations (locally adv./met.) **Dato-DXd** – TNBC (locally recurrent inop./met.) (TROPION-Breast02)	**tozorakimab** – acute respiratory failure (TILIA) ***Fasenra*** – CRwNP (ORCHID) ***Fasenra*** – bullous pemphigoid (FJORD) ***Tezspire*** – chronic rhinosinusitis with nasal polyps (WAYPOINT) ***Tezspire*** – severe asthma (DIRECTION) ***Ultomiris*** – DM (ALXN1210-DM-310) ***Koselugo*** – NF1-PN (KOMET) **acoramidis** – ATTR-CM (ALXN2060-TAC-302) **anselamimab** – AL amyloidosis (CAEL101-301 and -302)

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

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)	Dato-DXd (TROP2 ADC) volrustomig (PD-1/CTLA-4) capivasertib (AKT) camizestrant (ngSERD) rilvegostomig (PD-1/TIGIT) AZD5305 (PARP-1sel)	vemircopan (oral Factor D) gefurulimab (C5 mini-body) ALXN1850 (ngHPP)

Appendix: Glossary.

Movement since Q4 2022 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
	NME **AZD7789** PD1/TIM3 bispecific mAb solid tumours	Additional indication **Dato-DXd AVANZAR**[2] TROP2 ADC1L NSCLC, squamous and non-squamous 1L NSCLC, TROP2 BM+ **Dato-DXd TROPION Lung07**[2] TROP2 ADC 1L NSCLC PD-L1 <50% non-squamous **camizestrant CAMBRIA-1** selective estrogen receptor degrader HR+ HER2- extended adjuvant breast cancer Life-cycle management ***Tezspire* CROSSING#** TSLP mAb eosinophilic esophagitis ***Ultomiris* ARTEMIS** anti-complement C5 mAb cardiac surgery-associated acute kidney injury	**NME** **eplontersen# [US]**[1] ligand-conjugated antisense patients with hereditary transthyretin-mediated amyloid polyneuropathy (hATTR-PN) **danicopan [EU]** oral factor D inhibitor paroxysmal nocturnal haemoglobinuria with clinically significant extravascular haemolysis **Life-cycle management** ***Enhertu* DESTINY-Breast02# [EU]** HER2 targeting antibody drug conjugate HER2+, unresectable and/or metastatic breast cancer pre-treated with prior standard of care HER2 therapies, including T-DM1
Removed from Phase I	**Removed from Phase II**	**Removed from Phase III**	**Removed from registration**
NME **AZD8853#** GDF-15 solid tumours	**NME** cotadutide GLP-1/glucagon dual agonist non-alcoholic steatohepatitis **Life-cycle management** ***Imfinzi* (platform) MAGELLAN#** PD-L1 mAb + multiple novel oncology therapies +/- CTx 1st-line metastatic non-small cell lung cancer	**NME** **ALXN1840** bis-choline tetrathiomolybdate Wilson disease	

Phase progressions based on first patient dose achievement
#Partnered and/or in collaboration [1]Submission accepted [2]First patient dosed in Q1 2023
Appendix: Glossary.

Q1 2023 Oncology new molecular entity[1] pipeline

Phase I
6 New Molecular Entities

- AZD0466#
  BCL2/xL haematological malignancies
- AZD1390
  ATM glioblastoma
- AZD9574
  PARP inhibitor advanced solid malignancies
- AZD9592
  EGFR/cMET solid tumours
- IPH5201#
  CD39 solid tumours
- volrustomig + lenvatinib
  PD-1/CTLA-4+VEGF advanced RCC

Phase II
13 New Molecular Entities

- AZD0171 + *Imfinzi* + CTx
  anti-LIF+PD-L1+CTx 1L metastatic PDAC
- AZD4573
  CDK9 haematological malignancies
- AZD4573 + *Calquence*
  CDK9+BTK haematological malignancies
- 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
- oleclumab+CTx or *Imfinzi*+oleclumab+CTx
  CD73+chemo or PD-L1+CD73+chemo metastatic pancreatic cancer
- rilvegostomig (AZD2936) ARTEMIDE-01#
  PD1/TIGIT bispecific mAb solid tumours
- volrustomig
  PD-1/CTLA-4 solid tumours

Phase III
18 New Molecular Entities

- camizestrant + CDK4/6i SERENA-6
  SERD+CDK4/6 1L HR+ HER2- ESR1m breast cancer
- capivasertib + abiraterone CAPItello-281
  AKT+abiraterone PTEN deficient mHSPC
- capivasertib + docetaxel CAPItello-280
  AKT+Docetaxel mCRPC prostate cancer
- capivasertib + fulvestrant CAPItello-291
  AKT+fulvestrant 2L and beyond in AI resistant locally advanced or mBC
- ceralasertib + *Imfinzi* MONETTE
  ATR inhibitor + PDL-1 melanoma
- datopotamab deruxtecan TROPION-Breast02#
  TROP2 ADC 1L TNBC
- datopotamab deruxtecan TROPION-Lung01#
  TROP2 ADC 2L+ NSCLC with or without actionable genomic alterations
- datopotamab deruxtecan TROPION-Lung08#
  TROP2 ADC 1L metastatic NSCLC
- *Imfinzi* +/- oleclumab +/- monalizumab PACIFIC-9#
  PD-L1+NKG2A or PD-L1+CD73 unresectable stage III NSCLC
- camizestrant CAMBRIA-1
  SERD HR+ HER2- extended adjuvant breast cancer
- camizestrant + palbociclib SERENA-4
  SERD+CDK4/6 1L HR+ HER2- breast cancer
- capivasertib + CTx CAPItello-290
  AKT+chemotherapy 1L mTNBC
- capivasertib + fulvestrant + palbociclib CAPItello-292
  AKT+fulvestrant+CDK4/6 1L triplet in early relapse/ET resistant locally advanced or mBC
- ceralasertib + *Imfinzi* LATIFY
  ATR inhibitor + PDL-1 non-small cell lung cancer
- datopotamab deruxtecan TROPION-Breast01#
  TROP2 ADC 2-3L HR+ HER2- breast cancer
- datopotamab deruxtecan TROPION-Breast03#
  TROP2 ADC adjuvant residual disease TNBC
- datopotamab deruxtecan TROPION-Lung07#
  TROP 2 ADC 1L NSCLC PD-L1 <50% non-squamous
- datopotamab deruxtucan AVANZAR#
  TROP 2 ADC 1L NSCLC, squamous and non-squamous 1L NSCLC, TROP2 BM+

Under review
0 New Molecular Entities

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
Appendix: Glossary.

Precision medicine approach being explored

Q1 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 HER2 mutant 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 ONC therapies NSCLC
- *Imfinzi* (platform) HUDSON PD-L1+multiple novel ONC therapies post IO non-small cell lung cancer
- *Imfinzi* (platform) NeoCOAST# PD-L1+multiple novel ONC therapies NSCLC
- *Imfinzi* + *Lynparza* ORION# PD-L1+PARP 1L mNSCLC
- *Lynparza* (basket) LYNK002# PARP HRRm cancer
- *Tagrisso* + *Orpathys* SAVANNAH# EGFR+MET advanced EGFRm NSCLC
- *Tagrisso* ORCHARD platform study# EGFR+multiple novel ONC therapies 2L EGFRm osimertinib-resistant NSCLC

Phase III
33 Projects

- *Calquence* + R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL
- *Enhertu* DESTINY-Breast05# HER2 ADC HER2+ post-neoadjuvant high-risk breast cancer
- *Enhertu* DESTINY-Lung04# HER2 ADC HER2m NSCLC 1L
- *Imfinzi* + CRT PACIFIC-5 (China)# PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi* + domvanalimab (AB154) PACIFIC-8# PD-L1+TIGIT+CTx unresectable stage III NSCLC
- *Imfinzi* + FLOT MATTERHORN# PD-L1+CTx neo-adjuvant/adjuvant gastric cancer
- *Imfinzi* + VEGF EMERALD-2# PD-L1+VEGF adjuvant HCC
- *Imfinzi* post-SBRT PACIFIC-4# PD-L1 mAb post-SBRT stage I/II NSCLC
- *Lynparza* MONO-OLA1# PARP 1L BRCAwt ovarian cancer
- *Tagrisso* LAURA EGFR inhibitor stage III EGFRm NSCLC
- *Tagrisso* ADAURA2 adjuvant EGFRm NSCLC stage Ia2-Ia3 following complete tumour resection
- *Calquence* + venetoclax + obinutuzumab AMPLIFY# BTK+BCL-2+anti-CD20 1L CLL
- *Enhertu* DESTINY-Breast06# HER2 ADC post-ET HER2-low/HR+ breast cancer 2L
- *Enhertu* DESTINY-Gastric04# HER2 ADC HER2+ gastric 2L
- *Imfinzi* + CRT PACIFIC-2# PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi* + CTx NIAGARA PD-L1+CTx muscle invasive bladder cancer
- *Imfinzi* + *Imjudo* + SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer
- *Imfinzi* + VEGF + TACE EMERALD-1# PD-L1+VEGF+TACE locoregional HCC
- *Imfinzi* POTOMAC PD-L1 non-muscle invasive bladder cancer
- *Lynparza* + *Imfinzi* DUO-E# PARP+PD-L1 1L endometrial cancer
- *Tagrisso* + CTx FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC
- *Tagrisso* +/- CTx neoadjuvant NeoADAURA EGFR+/-CTx stage II/III resectable EGFRm NSCLC
- *Calquence* ECHO# BTK inhibitor 1L MCL
- *Enhertu* DESTINY-Breast11# HER2 ADC Neoadjuvant HER2+ breast cancer
- *Enhertu* DESTINY-Breast09# HER2 ADC HER2+ breast cancer 1L
- *Imfinzi* + CRT KUNLUN# PD-L1+CRT locally-advanced ESCC
- *Imfinzi* + CTx neoadjuvant AEGEAN PD-L1+CTx locally-advanced stage II-III NSCLC
- *Imfinzi* + EV +/- *Imjudo* VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 MIBC
- *Imfinzi* + *Imjudo* + TACE +/- lenvatinib EMERALD-3 PD-L1+CTLA4+VEGF+/-chemo-embolization locoregional HCC
- *Imfinzi* +/- *Imjudo* + CRT ADRIATIC# PD-L1+/-CTLA-4+CRT 1L LS-SCLC
- *Lynparza* + *Imfinzi* + bevacizumab DUO-O# PARP+PD-L1+VEGF 1L ovarian cancer
- *Orpathys* + *Imfinzi* SAMETA# MET+PD-L1 1L papillary renal cell carcinoma
- *Tagrisso* + *Orpathys* SAFFRON# EGFR + MET advanced EGFRm non-small cell lung cancer

Under review
1 Project

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

Phase progressions based on first patient dose achievement
[1]Includes significant life-cycle management projects and parallel indications for assets beyond Phase III
#Partnered and/or in collaboration; ¶Registrational Phase II/III trial
Appendix: Glossary.

● Precision medicine approach being explored

Q1 2023 BioPharmaceuticals new molecular entity[1] pipeline

Phase I
17 New Molecular Entities

- AZD0186
  GLP1R agonism type-2 diabetes
- AZD2373
  podocyte health nephropathy
- AZD3366
  CD39L3 cardiovascular disease
- AZD4041#
  orexin 1 receptor antagonist opioid use disorder
- AZD5055
  Porcupine inhibitor idiopathic pulmonary fibrosis
- AZD6234
  peptide obesity with related comorbidities
- AZD7503
  ASO non-alcoholic steatohepatitis
- AZD8630#
  Inhaled TSLP FAb asthma
- MEDI1814#
  amyloid beta mAb alzheimer's disease
- AZD0780
  PCSK9 dyslipidemia
- AZD2693
  NASH resolution non-alcoholic steatohepatitis
- AZD3427
  Relaxin mimetic heart failure
- AZD4604
  inhaled JAK1 asthma
- AZD5462
  RXFP1 agonist CV disease
- AZD6793
  IRAK4 inhibitor inflammatory diseases
- AZD7798
  humanised monoclonal antibody targets T cells subset Crohn's disease
- MEDI0618*
  PAR2 antagonist osteoarthritis pain

Phase II
12 New Molecular Entities

- atuliflapon
  FLAP asthma
- balcinrenone/dapagliflozin
  MR+SGLT2 heart failure with CKD
- brazikumab EXPEDITION
  IL-23 mAb ulcerative colitis
- elarekibep (AZD1402)#
  Inhaled IL-4Ra asthma
- MEDI1341#
  alpha synuclein mAb multiple system atrophy/parkinson's disease
- MEDI6570
  LOX-1 CV disease
- MEDI7352
  NGF/TNF OA pain / PDN
- mitiperstat
  myeloperoxidase COPD
- mitiperstat
  MPO HFpEF / NASH
- tozorakimab
  IL-33 diabetic kidney disease
- tozorakimab FRONTIER 3
  IL-33 asthma
- zibotentan/dapagliflozin
  endothelin A receptor antagonist + SGLT2 CKD/ liver cirrhosis

Phase III
5 New Molecular Entities

- AZD3152 SUPERNOVA¶
  SARS-CoV-2 LAAB prevention of COVID-19
- brazikumab INTREPID
  IL-23 Crohn's disease
- eplontersen#
  LICA ATTR-Cardiomyopathy
- tozorakimab OBERON TITANIA PROSPERO
  IL-33 COPD
- tozorakimab TILIA
  IL-33 mAb 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
Appendix: Glossary.

● Precision medicine approach being explored

Q1 2023 BioPharmaceuticals lifecycle management[1] pipeline

Phase I
0 Projects

Phase II
3 Projects

- *Andexxa*
  anti-factor Xa reversal urgent surgery
- roxadustat#
  HIFPH anaemia chemotherapy induced anaemia
- *Tezspire* COURSE#
  TSLP chronic obstructive pulmonary disease

Phase III
14 Projects

- *Breztri/Trixeo* (PT010) KALOS LOGOS
  LABA/LAMA/ICS asthma
- *Fasenra* RESOLUTE#
  IL-5R chronic obstructive pulmonary disease
- *Fasenra* MANDARA
  IL-5R eosinophilic granulomatosis with polyangiitis
- *Fasenra* ORCHID#
  IL-5R nasal polyps
- *Lokelma* STABILIZE-CKD
  potassium binder hyperkalaemia in CKD
- *Saphnelo* IRIS#
  Type I IFN receptor mAb lupus nephritis
- *Tezspire* WAYPOINT#
  TSLP nasal polyps
- *Farxiga/Forxiga* DAPA-MI
  SGLT-2 prevention of HF and CV death following a myocardial infarction
- *Fasenra* FJORD
  IL-5R bullous pemphigoid
- *Fasenra* NATRON
  IL-5R hypereosinophilic syndrome
- *Lokelma* DIALIZE-Outcomes
  potassium binder CV outcomes in patients on chronic haemodialysis with hyperkalaemia
- roxadustat#
  HIFPH anaemia MDS
- *Saphnelo* TULIP-SC#
  Type I IFN receptor SLE SC
- *Tezspire* CROSSING#
  TSLP eosinophilic esophagitis

Under review
0 Projects

Phase progressions based on first patient dose achievement
[1]Includes significant life-cycle management projects and parallel indications for assets beyond Phase III
#Partnered and/or in collaboration; ¶Registrational Phase II/III trial
Appendix: Glossary.

Precision medicine approach being explored

Q1 2023 Rare Disease pipeline[1]

Phase I
6 Projects

- ALXN1850
  next gen TNSALP ERT hypophosphatasia
- ALXN1910
  next gen TNSALP ERT bone metabolism
- ALXN2030
  siRNA targeting complement C3 nephrology
- ALXN2080
  oral factor D healthy volunteers
- ALXN2220 (NI006)#
  TTR depleter transthyretin amyloid cardiomyopathy
- tarperprumig (ALXN1820)
  anti-properdin bi-specific haematology

Phase II
5 Projects

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

Phase III
5 Projects

- acoramidis#
  oral TTR stabilizer transthyretin amyloid cardiomyopathy
- anselamimab (CAEL-101)
  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
2 Projects

- danicopan
  factor D PNH with clinically significant extravascular haemolysis
- *Ultomiris* CHAMPION-NMOSD
  anti-complement C5 mAb neuromyelitis optica spectrum disorder

Phase progressions based on first patient dose achievement
[1]Includes new molecular entities and significant life-cycle management projects
#Partnered and/or in collaboration; ¶Registrational Phase II/III trial
Appendix: Glossary.

Precision medicine approach being explored

Estimated key regulatory submission acceptances

	H1 2023	H2 2023	2024	>2024	
NME	capivasertib + fulvestrant CAPItello-291 2L and beyond in AI resistant locally advanced or mBC	AZD3152 SUPERNOVA prevention of COVID-19	anselamimab (CAEL-101) AL amyloidosis	capivasertib + fulvestrant + palbociclib CAPItello-292 1L triplet in early relapse/ET resistant locally advanced or mBC	datopotamab deruxtecan TROPION-Lung08 1L metastatic NSCLC	gefurulimab generalised myasthenia gravis
		datopotamab deruxtecan TROPION-Lung01 2L+ NSCLC with or without actionable genomic alterations	acoramidis transthyretin amyloid cardiomyopathy	capivasertib + docetaxel CAPItello-280 mCRPC prostate cancer	datopotamab deruxtecan TROPION-Lung07 1L NSCLC PD-L1 <50% non-squamous	tozorakimab OBERON TITANIA PROSPERO COPD
			tozorakimab TILIA Acute Respiratory Failure	capivasertib + abiraterone CAPItello-281 PTEN deficient mHSPC	datopotamab deruxtecan TROPION-Breast03 adjuvant residual disease TNBC	eplontersen ATTR-Cardiomyopathy
				camizestrant CAMBRIA-1 HR+ HER2- extended adjuvant breast cancer	datopotamab deruxtecan TROPION-Breast02 1L TNBC	brazikumab INTREPID Crohn's disease
		datopotamab deruxtecan TROPION-Breast01 2-3L HR+ HER2- breast cancer	capivasertib + CTx CAPItello-290 1L mTNBC	camizestrant + palbociclib SERENA-4 1L HR+ HER2- breast cancer	ceralasertib + *Imfinzi* MONETTE melanoma	*Imfinzi* +/- oleclumab +/- monalizumab PACIFIC-9 unresectable stage III NSCLC
				camizestrant + CDK4/6i SERENA-6 1L HR+ HER2- ESR1m breast cancer	ceralasertib + *Imfinzi* LATIFY non-small cell lung cancer	datopotamab deruxtucan AVANZAR 1L NSCLC, squamous and non-squamous 1L NSCLC, TROP2 BM+
LCM		*Imfinzi* + CTx neoadjuvant AEGEAN locally-advanced stage II-III NSCLC	*Calquence* ECHO 1L MCL	*Calquence* + R-CHOP ESCALADE 1L DLBCL	*Imfinzi* post-SBRT PACIFIC-4 stage I/II NSCLC	*Tezspire* WAYPOINT nasal polyps
		Imfinzi + CTx NIAGARA muscle invasive bladder cancer	*Enhertu* DESTINY-Breast11 Neoadjuvant HER2+ breast cancer	*Calquence* + venetoclax + obinutuzumab AMPLIFY 1L CLL	*Imfinzi* POTOMAC non-muscle invasive bladder cancer	*Tezspire* CROSSING eosinophilic esophagitis
		Imfinzi + *Imjudo* + SoC NILE 1L urothelial cancer	*Enhertu* DESTINY-Breast06 post-ET HER2-low/HR+ breast cancer 2L	*Enhertu* DESTINY-Breast05 HER2+ post-neoadjuvant high-risk breast cancer	*Lynparza* MONO-OLA1 1L BRCAwt ovarian cancer	*Ultomiris* dermatomyositis
		Tagrisso LAURA stage III EGFRm NSCLC	*Imfinzi* + CRT PACIFIC-2 locally-advanced stage III NSCLC	*Enhertu* DESTINY-Breast09 HER2+ breast cancer 1L	*Orpathys* + *Imfinzi* SAMETA 1L papillary renal cell carcinoma	*Ultomiris* haematopoietic stem cell transplant–associated thrombotic microangiopathy
		Tagrisso + CTx FLAURA2 1L adv EGFRm NSCLC	*Imfinzi* + VEGF + TACE EMERALD-1 locoregional HCC	*Enhertu* DESTINY-Gastric04 HER2+ gastric 2L	*Tagrisso* + *Orpathys* SAFFRON advanced EGFRm non-small cell lung cancer	*Ultomiris* ARTEMIS cardiac surgery-associated acute kidney injury
			Imfinzi +/- *Imjudo* + CRT ADRIATIC 1L LS-SCLC	*Enhertu* DESTINY-Lung04 HER2m NSCLC 1L	*Tagrisso* ADAURA2 adjuvant EGFRm NSCLC stage Ia2-Ia3 following complete tumour resection	
			Lynparza + *Imfinzi* + bevacizumab DUO-O 1L ovarian cancer	*Imfinzi* + CRT KUNLUN locally-advanced ESCC	*Breztri/Trixeo* (PT010) KALOS LOGOS asthma	
			Lynparza + *Imfinzi* DUO-E 1L endometrial cancer	*Imfinzi* + CRT PACIFIC-5 (China) locally-advanced stage III NSCLC	*Fasenra* RESOLUTE chronic obstructive pulmonary disease	
			Tagrisso +/- CTx neoadjuvant NeoADAURA stage II/III resectable EGFRm NSCLC	*Imfinzi* + domvanalimab (AB154) PACIFIC-8 unresectable stage III NSCLC	*Fasenra* ORCHID nasal polyps	
			Fasenra FJORD bullous pemphigoid	*Imfinzi* + EV +/- *Imjudo* VOLGA MIBC	*Lokelma* DIALIZE-Outcomes CV outcomes in patients on chronic haemodialysis with hyperkalaemia	
			Fasenra MANDARA eosinophilic granulomatosis with polyangiitis	*Imfinzi* + FLOT MATTERHORN neo-adjuvant/adjuvant gastric cancer	Lokelma STABILIZE-CKD hyperkalaemia in CKD	
			Fasenra NATRON hypereosinophilic syndrome	*Imfinzi* + *Imjudo* + TACE +/- lenvatinib EMERALD-3 locoregional HCC	*Saphnelo* IRIS lupus nephritis	
			roxadustat anaemia MDS	*Imfinzi* + VEGF EMERALD-2 adjuvant HCC	*Saphnelo* TULIP-SC SLE SC	

Oncology BioPharmaceuticals Rare Disease

NME section includes additional indications for assets where the lead is not yet launched

Appendix: Glossary.

Designations in our pipeline

3 Accelerated approvals

- *Andexxa* Acute Major Bleed (US)
- *Calquence* MCL 1L (US)
- *Beyfortus* RSV mAb-YTE (EU)

14 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+ breast 2L DESTINY-Breast03 (US)
- *Enhertu* HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
- *Enhertu* HER2+/HER2-low gastric 3L DESTINY-Gastric01 (JP)[2]
- *Enhertu* HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
- *Enhertu* HER2-low unresectable and/or metastatic breast cancer DESTINY-Breast04 (US)
- *Koselugo* NFI type 1 SPRINT (US)
- *Tezspire* asthma NAVIGATOR (US)

9 Fast Track

- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- anselamibab (CAEL-101) AL amyloidosis (US)
- camizestrant 1L HR+ HER2- ESR1m breast cancer SERENA-6 (US)
- capivasertib+fulv HR+ breast 2L+ (CAPItello-291)
- *Forxiga* MI RRCT DAPA-MI (US)
- *Lokelma* ESRD DIALIZE-OUTCOMES (US)
- *Saphnelo* SLE (US)
- tozorakimab acute respiratory failure (US)
- *Orpathys* + *Tagrisso* NSCLC (SAVANNAH / SAFFRON) (US)

14 Priority Review

- *Calquence* MCL 1L (US)
- *Enhertu* HER2+ breast 2L DESTINY-Breast03 (US)
- *Enhertu* HER2+ breast 2L DESTINY-Breast03 (CN)
- *Enhertu* HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
- *Enhertu* HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
- *Enhertu* HER2-low unresectable and/or metastatic breast cancer DESTINY-Breast04 (US)
- *Imfinzi* + CTx BTC 1L (TOPAZ-1) (US)
- *Imfinzi* + *Imjudo* HCC 1L (HIMALAYA) (US)
- *Koselugo* NFI type 1 SPRINT (US)
- *Lynparza* + abiraterone all-comers mCRPC 1L (PROpel)
- *Lynparza* gBRCA adj breast OlympiA (US)
- *Roxadustat* chronic kidney disease (CN)
- *Tezspire* asthma NAVIGATOR (US)
- *Ultomiris* gMG (US)

26 Orphan

- *Andexxa* Acute Major Bleed (JP)
- anselamibab (CAEL-101) AL amyloidosis (US)
- anselamibab (CAEL-101) 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* NFI type 1 SPRINT (US)
- *Koselugo* NFI type 1 SPRINT (EU)
- *Koselugo* NFI type 1 SPRINT (JP)
- *Lynparza* gBRCA adj breast OlympiA (JP)
- *Tezspire* eosinophilic esophagitis (US)
- *Ultomiris* DM (US)
- *Ultomiris* HSCT-TMA (US)
- *Ultomiris* SC 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.

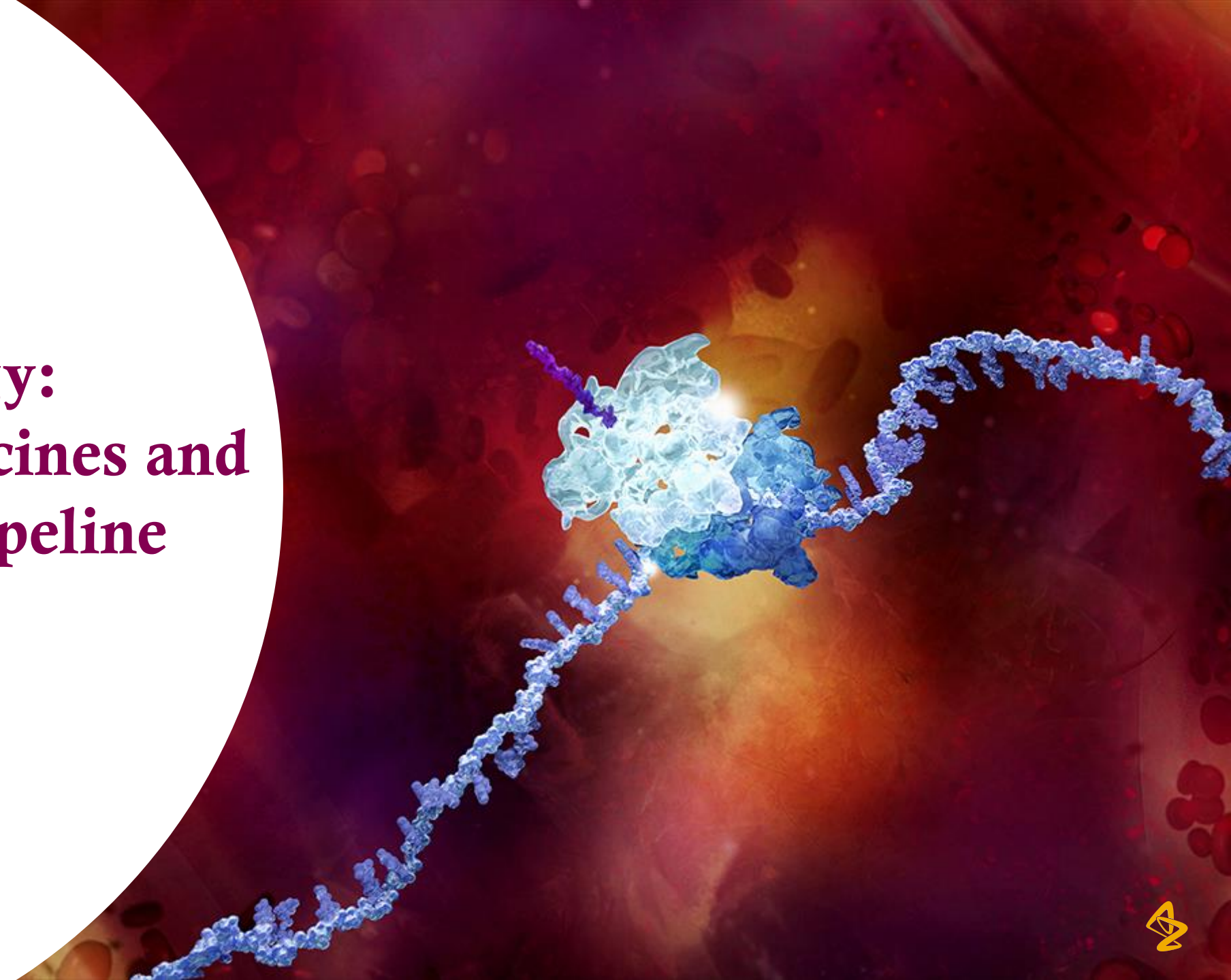
ORPHAN DRUG DESIGNATION, intended for treatment, diagnosis or prevention of rare diseases/disorders that affect fewer than 200,000 patients in the US, or that affect more than 200,000 patients but are not expected to recover the costs of developing and marketing a treatment drug.

Oncology BioPharmaceuticals Rare Disease

Excludes designations for projects which have launched in all applicable major markets

Appendix: Glossary.

Oncology: approved medicines and late-stage pipeline



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III ADAURA NCT02511106	Adjuvant EGFRm NSCLC	682	• Arm 1: *Tagrisso* QD following complete tumour resection, with or without chemotherapy • Arm 2: placebo • Global trial – 25 countries	• Primary endpoint: DFS • Secondary endpoints: DFS Rate, OS, OS rate and QoL	• FPCD: Q4 2015 • LPCD: Q1 2019 • Data readout: Q2 2020 • Trial unblinded due to efficacy; primary and secondary (OS) endpoints met
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: H2 2023
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

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: 2024
Phase III FLAURA2 NCT04035486	1L EGFRm NSCLC	586	• Arm 1: *Tagrisso* plus 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 anticipated: H1 2023
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: 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: 2024

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: 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: 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

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: 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 • 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: >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 A: TACE + T300 + D + lenvatanib • Arm B: TACE + T300 + D • Arm C: TACE	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q2 2022 • Data anticipated: >2024

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: 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: 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

Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
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
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	340	• Open-label, biomarker-directed, multicentre 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 COAST NCT03822351	Stage III unresectable NSCLC	189	• Arm A: *Imfinzi* • Arm B: *Imfinzi* + oleclumab • Arm C: *Imfinzi* + monalizumab	• Primary endpoint: OR per RECIST v1.1	• FPCD: Q4 2018 • Data readout: Q3 2021
Phase II NeoCOAST NCT03794544	Resectable, early-stage NSCLC	84	• Arm A: *Imfinzi* • Arm B: *Imfinzi* + oleclumab • Arm C: *Imfinzi* + monalizumab • Arm D: *Imfinzi* + danvatirsen	• Primary endpoint: major pathological response rate	• FPCD: Q1 2019 • LPCD: Q1 2021 • Data readout: Q1 2022

Appendix: Glossary.

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II MAGELLAN NCT03819465	1L NSCLC	212	• Arm A1: *Imfinzi* • Arm A2: *Imfinzi* + danvatirsen • Arm A3: *Imfinzi* + oleclumab • Arm A4: MEDI5752 • Arm A5: rilvegostomig • Arm B1: *Imfinzi* + Investigator's choice of chemo • Arm B2: *Imfinzi* + danvatirsen + Investigator's choice of chemo • Arm B3: *Imfinzi* + oleclumab + Investigator's choice of chemo • Arm B4: MEDI5752 • Arm B5: rilvegostomig + chemotherapy	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, DoR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2019 • Trial discontinued due to strategic portfolio prioritisation
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: MEDI5752 + 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, multicentre trial to evaluate the safety, PK and preliminary efficacy of s.c. *Imfinzi*	• Primary endpoints: PK parameters and safety	• FPCD: Q4 2021 • Data anticipated: H2 2023

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: H2 2023
Phase III SAMETA NCT05043090	MET-driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	• *Orpathys* + *Imfinzi* vs. sunitinib and *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: H2 2023
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	210	• Arm 1: *Imfinzi* + paclitaxel • Arm 2: *Imfinzi* + paclitaxel + capivasertib • Arm 5: *Imfinzi* + paclitaxel + oleclumab • Arm 6: *Imfinzi* + *Enhertu* • Arm 7: *Imfinzi* + datopotamab deruxtecan • Global trial	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, PFS, DoR, OS, PK and ADA	• FPCD: Q1 2019 • Data anticipated: H1 2023
Phase I CLOVER NCT03509012	HNSCC, NSCLC, SCLC	167	• *Imfinzi* +/- *Imjudo* in combination with chemoradiation in advanced solid tumours	• Primary endpoint: safety	• FPCD: Q2 2018 • Dara readout Q4 2021

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: bevacizumab • Arm 2: bevacizumab + *Imfinzi* • Arm 3: bevacizumab + *Imfinzi* + *Lynparza* • tBRCAm patients • 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 and *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 anticipated: H2 2023

Appendix: Glossary.

Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered (BIG and NRG Oncology)	BRCAm 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* • Arm 2: placebo • Global trial – 12 countries	• Primary endpoints: PFS (BRCAwt HRD+ve) and PFS (BRCAwt) • Secondary endpoints: OS, TFST and PFS2	• FPCD: Q3 2021 • Data anticipated: 2024

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	680	• 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 anticipated: H2 2023
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

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 • *Enhertu* • 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 • *Enhertu* • 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 • *Enhertu* • 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 • *Enhertu* • 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 • *Enhertu* • Investigator's choice SoC chemotherapy (capecitabine, paclitaxel, nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q3 2020 • Data anticipated: H2 2023
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 • *Enhertu* + placebo • *Enhertu* + pertuzumab • SoC	• Primary endpoint: PFS • Secondary endpoints: OS, DoR and ORR	• FPCD: Q2 2021 • Data anticipated: >2024

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 • *Enhertu* • *Enhertu* followed by THP • doxorubicin and cyclophosphamide followed by THP	• Primary endpoint: pCR • Secondary endpoints: EFS, IDFS and OS	• FPCD: Q4 2021 • Data anticipated: 2024
Phase Ib/II **DESTINY-Breast07** **NCT04538742** **Partnered (Daiichi Sankyo)**	HER2-positive metastatic breast cancer	450	• Randomised, open-label, sequential assignment • *Enhertu* • *Enhertu* + *Imfinzi* • *Enhertu* + pertuzumab • *Enhertu* + paclitaxel • *Enhertu* + *Imfinzi* + paclitaxel • *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 • *Enhertu* + capecitabine • *Enhertu* + *Imfinzi* + paclitaxel • *Enhertu* + capivasertib • *Enhertu* + anastrozole • *Enhertu* + Faslodex	• Primary endpoints: AE and SAE • Secondary endpoints: ORR, PFS, DoR and OS	• FPCD: Q1 2021 • Data anticipated: H2 2023

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 • *Enhertu* • 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 • *Enhertu* • 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

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-Lung01** **NCT03505710** **Partnered (Daiichi Sankyo)**	HER2-overexpressing or mutated, unresectable and/or metastatic NSCLC	181	• Non-randomised, parallel group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, PFS, OS and DCR	• FPCD: Q2 2018 • LPCD: Q1 2022 • Data readout: Q3 2021 • Primary endpoint met
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 anticipated: H1 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

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: 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
Phase Ib U105 NCT03523572 Partnered (Daiichi Sankyo)	HER2-expressing breast and urothelial cancer	99	• Non-randomised, sequential assignment • *Enhertu* + nivolumab • Global trial – 7 countries	• Primary endpoints: DLT, ORR and TEAEs • Secondary endpoints: DoR, DCR, PFS, TTR, OS and ORR (investigator)	• FPCD: Q3 2018 • Data readout: Q3 2021

Appendix: Glossary.

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III ELEVATE-TN (ACE-CL-007) NCT02475681	Previously untreated CLL	535	• Arm A: chlorambucil + obinutuzumab • Arm B: *Calquence* + obinutuzumab • Arm C: *Calquence*	• Primary endpoint: PFS (Arm A vs. Arm B) • Secondary endpoints: IRC-assessed ORR and OS (Arm A vs. Arm B vs. Arm C)	• FPCD: Q2 2015 • Data readout: Q2 2019 • Primary endpoint met
Phase III AMPLIFY (ACE-CL-311) NCT03836261	Previously untreated CLL	981	• Arm A; *Calquence* + venetoclax • Arm B: *Calquence* + venetoclax + obinutuzumab • Arm C: FCR or BR	• Primary endpoint: IRC PFS (Arm A vs. Arm C) • Secondary endpoints: IRC PFS (Arm B vs. Arm C) and INV PFS (Arm A vs. Arm C; Arm B vs. Arm C)	• FPCD: Q1 2019 • Data anticipated: >2024
Phase III ASCEND (ACE-CL-309) NCT02970318	R/R CLL	306	• Arm A: *Calquence* • Arm B: rituximab + idelalisib or bendamustine (investigator's choice)	• Primary endpoint: IRC assessed PFS (Arm A vs. Arm B) • Secondary endpoints: INV-assessed ORR, OS, DoR and PROs	• FPCD: Q4 2017 • Data readout: Q2 2019 • Primary endpoint met
Phase III ELEVATE-RR (ACE-CL-006) NCT02477696	R/R high-risk CLL	533	• Arm A: *Calquence* • Arm B: ibrutinib	• Primary endpoint: PFS • Secondary endpoints: comparison of incidence of infections, RTs and atrial fibrillation, OS	• FPCD: Q3 2015 • Data readout: Q1 2021 • Primary endpoint met
Phase III ECHO (ACE-LY-308) NCT02972840	Previously untreated MCL	634	• Arm A: *Calquence* + bendamustine + rituximab • Arm B: 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 A: *Calquence* • Arm B: Chlorambucil + rituximab	• Primary endpoint: PFS • Secondary endpoints: ORR and DoR	• FPCD: Q1 2020 • Data anticipated: H2 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 rituximab • Arm A: treatment naive • Arm B: R/R • Arm C: treatment naïve: *Calquence* + venetoclax + rituximab	• Primary endpoint: safety	• FPCD: Q2 2016 • LPCD: Q2 2022 • Data readout: Q1 2023
Phase I/II **ACE-CL-001** **NCT02029443**	CLL, SLL, Richter's transformation	306	• *Calquence* monotherapy • Dose escalation and expansion	• Primary endpoints: safety, PK and PD parameters	• FPCD: Q1 2014 • Data readout: Q4 2021
Phase I **ACE-LY-003** **NCT02180711**	R/R follicular lymphoma	89	• Arm A: *Calquence* • Arm B: *Calquence* + rituximab • Arm C: *Calquence* + rituximab + lenolidomide	• Primary endpoint: safety	• FPCD: Q1 2015 • Data anticipated: 2024
Phase I **ACE-CL-003** **NCT02296918**	CLL/SLL/PLL	114	• *Calquence* + obinutuzumab • Arm A: R/R • Arm B: treatment naïve • *Calquence* + venetoclax + rituximab • Arm C: R/R • Arm D: treatment naïve	• Primary endpoints: safety and ORR • Secondary endpoints: PD, PFS, TTNT and OS	• FPCD: Q4 2014 • Data readout: Q1 2022

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: 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, multicentre, open-label trial • *Orpathys*	• Primary endpoint: ORR • Secondary endpoints: PFS and safety	• FPCD: Q3 2021 • Data anticipated: 2024

Appendix: Glossary.

capivasertib (AKT inhibitor)

Breast cancer, prostate cancer and indolent non-hodgkin lymphoma

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 • Data anticipated: H2 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 anticipated: H1 2023
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 2: pembrolizumab +pemetrexed • 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 + durvalumab • Arm 2: pembrolizumab • Global trial •	• Co-primary endpoints: OS and PFS in TROP2 biomarker-positive	• FPCD: Q1 2023 • Data anticipated: >2024
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 readout: Q1 2023
Phase I TROPION-Lung02 NCT04526691 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	140	• Open-label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy • US and Japan	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q4 2020 • Data anticipated: 2024

Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
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 + AZ2936 • Cohort 7 & 8: datopotamab deruxtecan + AZ2936 + carboplatin • Cohort 9 &10: datopotamab deruxtecan + MEDI5752 + Carboplatin • Cohort 11: datopotamab deruxtecan + MEDI5752 • US and Japan	• Primary endpoints: DLT and safety • Secondary endpoints: ORR, DOR, PFS, OS, PK parameters and ADA	• FPCD: Q1 2021 • Data anticipated: >2024
Phase I **TROPION-PanTumor01** **NCT03401385** **Partnered (Daiichi Sankyo)**	Subjects with advanced solid tumours: NSCLC, TNBC, HR+ breast cancer, HER2-negative gastric/GEJ, esophageal, urothelial, SCLC	770	• 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: 2024 • Early data readout (NSCLC) Q1 2021 • Early data readout (TNBC) Q2 2021
Phase II **TROPION-PanTumor03** **NCT05489211** **Partnered (Daiichi Sankyo)**	Endometrial cancer, gastric cancer, mCRPC, ovarian cancer, CRC	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 + durvalumab + 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 (cvarian 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	• Primary endpoints: ORR and safety	• FPCD: Q3 2022 • Data anticipated: >2024

Appendix: Glossary.

datopotamab deruxtecan (TROP2 ADC)

Breast cancer

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 • datopotamab deruxtecan vs. investigator's choice SoC chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine)	• Primary endpoints: PFS and OS • Secondary endpoints: ORR, DoR, 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 • datopotamab deruxtecan vs. 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: 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

Appendix: Glossary.

camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2- breast cancer	1342	• Randomised, double-blind, comparative trial • Arm A: camizestrant + palbociclib • Arm B: anastrazole + palbociclib	• Primary endpoint: PFS • Secondary endpoints: OS and PFS2	• FPCD: Q1 2021 • Data anticipated: >2024
Phase III SERENA-6 NCT04964934	HR+ HER2- breast cancer	300	• Randomised, double-blind, comparator trial • Arm A: camizestrant + palbociclib or abemaciclib • Arm B: 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 A: continue standard ET of investigator's choice • Arm B: 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+ breast cancer	240	• Randomised, open-label, parallel-group, multicentre trial • camizestrant vs. i.m. Faslodex in women with advanced breast cancer	• Primary endpoint: mPFS	• FPCD: Q2 2020 • LPCD: Q3 2021 • Data readout: Q4 2022 • Primary endpoint met at 75mg and 150mg doses
Phase II SERENA-3 NCT04588298	HR+ breast cancer	132	• Randomised, open-label, parallel-group, multicentre trial	• Primary endpoint: change in ER expression between pre- and on-treatment tumour biopsies	• FPCD: Q4 2020 • Data anticipated: H1 2023

Appendix: Glossary.

camizestrant (AZD9833, next-generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04541433**	HR+ breast cancer	18	• Open-label trial • Anti-tumour activity of camizestrant in Japanese women with endocrine resistant HR+ HER2- breast cancer that is not amenable to treatment with curative intent	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2020 • LPCD: Q1 2022 • Data readout: Q1 2023
Phase I **SERENA-1** **NCT03616587**	HR+ breast cancer	403	• Escalation phase: open-label multicentre 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: 2024
Phase I **NCT04818632**	HR+ HER2- breast cancer in Chinese patients	30	• Dose escalation: camizestrant • Dose expansion: • Cohort 1: camizestrant • Cohort 2: camizestrant + palbociclib • Cohort 3: camizestrant + everolimus	• Primary endpoints: safety and tolerability, PK parameters • Secondary endpoint: anti-tumour activity	• FPCD: Q1 2021 • LPCD: Q1 2023 • Data anticipated: H2 2023

Appendix: Glossary.

ceralasertib (AZD6738, ATR inhibitor)

Cancer

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 PLANETTE NCT04564027	Solid tumours, mCRPC	61	• Cohort A: ceralasertib; ATM-altered AST • Cohort B: ceralasertib; ATM-altered mCRPC • North America and Europe	• Primary endpoint (Cohort A): ORR • Primary endpoint (Cohort B): CRR	• FPCD: Q1 2021 • Trial discontinued due to strategic portfolio prioritisation
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: 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 • North America, Europe and South Korea	• Primary endpoints: safety and tolerability, efficacy and PK parameters	• FPCD: Q4 2014 • Data anticipated: >2024

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 of AZD0171 + *Imfinzi* + gemcitabine, nab-paclitaxel	• Primary endpoints: safety, OS at 12 months • Secondary endpoints: ORR, DoR, PFS	• FPCD: Q1 2022 • Data anticipated: 2024

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 • Data anticipated: 2024
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 • Data anticipated: >2024

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 A: recurrent GBM, AZD1390 + RT in dose escalation cohorts • Arm C: 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: 2024

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 study in pts with R/R PTCL or cHL • Module 1: AZD4573 monotherapy • Cohort 1: PTCL, all comers (excluding NKTCL) • Cohort 2: PTCL (NKTCL only) • Cohort 3: cHL • i.v. route of administration • Global trial – 30 countries	• Primary endpoint: efficacy • Secondary endpoints: safety and PK parameters	• FPCD: Q4 2021 • Data anticipated: 2024
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 • Data anticipated: 2024
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 • Netherlands, UK, Germany	• Primary endpoints: safety and PK parameters • Secondary endpoint: efficacy	• FPCD: Q4 2017 • LPCD: Q3 2021 • Data readout: Q4 2022

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, multicentre 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 study 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

Appendix: Glossary.

AZD7789 (PD-1/TIM3 bispecific mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa NCT04931654	NSCLC, other tumours	81	• Open-label, non-randomised dose-escalation and dose-expansion trial • Part A: dose escalation in post-IO NSCLC patients with AZD7789 i.v. monotherapy • Part B: dose expansion in post-IO and IO-naïve NSCLC patients with AZD7789 i.v. monotherapy • North America, Europe	• 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 AZD7789 • Cohort B1: dose expansion where patients with anti-PD-1/PD-L1 exposed R/R cHL will receive AZD7789 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 AZD7789 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

Appendix: Glossary.

AZD8205 (B7H4 ADC)

Cancer

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	• 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

Appendix: Glossary.

AZD8853 (anti-GDF15)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05397171	Selected, advanced metastatic solid tumours	165	• Open-label trial • AZD8853 monotherapy • Part A: dose escalation, • Part B: safety expansion/proof of mechanism utilising exploratory CD8+ PET imaging • Part C: efficacy expansion	• Primary endpoints: safety and tolerability • Secondary endpoints: ORR, DCR, DoR, PFS, PK parameters, PD and immunogenicity	• FPCD: Q2 2022 • Trial discontinued due to strategic portfolio prioritisation

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, dose escalation trial	• 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

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, multicentre trial • AZD9592 • AZD9592 + osimertinib • Expansion phase, open-label, multicentre trial • AZD9592 • AZD9592 + osimertinib	• 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

Appendix: Glossary.

capivasertib (AKT inhibitor)

Breast cancer, prostate cancer and indolent non-hodgkin lymphoma

Trial	Population	Patients	Design	Endpoints	Status
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 • Data anticipated: H2 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. • 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: Q4 2022

Appendix: Glossary.

oleclumab (CD73 mAb)

Cancer

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. • 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 • Data anticipated: H1 2023

Appendix: Glossary.

rilvegostomig (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 AZD2936 i.v. monotherapy • Part B: dose expansion in CPI-experienced NSCLC patients with AZD2936 i.v. monotherapy • Part C: dose expansion in CPI-naive NSCLC patients with AZD2936 i.v. monotherapy • Part D: randomised dose expansion in CPI-naive NSCLC patients with AZD2936 i.v. monotherapy • 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: 2024

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	• Multicentre, Phase I, open-label, dose-escalation and expansion study	• 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

Appendix: Glossary.

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

Cancer

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 A: volrustomig i.v. • Arm B: volrustomig i.v., pemetrexed and carboplatin • Arm C: pembrolizumab, pemetrexed and carboplatin • Arm D: 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

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: 2024
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

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 1000 mgXR • 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

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 **HARMONIZE Asia** **NCT03528681**	Hyperkalaemia	250	• Open-label *Lokelma* 10g TID for 48 hours followed by: • Arm 1: *Lokelma* 5g QD for 28 days • Arm 2: *Lokelma* 10g QD for 28 days • Arm 3: placebo QD for 28 days • China only	• Primary endpoint: maintenance of normokalaemia	• FPCD: Q2 2021 • LPCD: Q3 2022 • Data readout: Q4 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

Appendix: Glossary.

roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III 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 anticipated: H1 2023
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 • Data anticipated: H1 2023

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: change in baselines from 6MWT and 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 (hATTR-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 • 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 • Secondary endpoints: changes from baseline in Norfolk QOL-DN at Week 35, NSC score at Weeks 35 and 66, PCS score of the SF-36 at Week 65, PND score at Week 65 and mBMI at Week 65	• FPCD: Q1 2020 • Data readout: Q2 2022 • Co-primary endpoints met

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: efficacy and safety	• FPCD: Q3 2021 • Data anticipated: >2024

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 A: tozorakimab dose 1 + *Farxiga* • Arm B: tozorakimab dose 2 + *Farxiga* • Arm C: tozorakimab dose 3 + *Farxiga* • Arm D: tozorakimab dose 4 + *Farxiga* • Arm E: placebo + *Farxiga* • US, Canada, Japan and additional countries	• Primary endpoint: UACR • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2019 • LPCD: Q3 2022 • Data anticipated: H2 2023

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 study • US only	• Primary endpoints: PK parameters	• FPCD: Q3 2022 • Data anticipated: H1 2023

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 multicentre, randomised, double-blind, placebo-controlled, parallel group dose-ranging study • 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: 2024

Appendix: Glossary.

Breztri (next-generation propellant)

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, multicentrestudy • 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: 2024
Phase I NCT05477108	Healthy volunteers	108	• Randomised, double-blind, single-dose, single-center, partial-replicate, 3-way cross-over • 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-center, partial-replicate, 3-way cross-over study • 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: 2024

Appendix: Glossary.

Breztri, *Trixeo* (PT010, LAMA/LABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III KALOS NCT04609878	Severe asthma	2200	• Randomised, double-blind, double-dummy, parallel group and multicentre 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 multicentre 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, multicentre 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: 2024
Phase III LITHOS NCT05755906	Mild to moderate asthma	340	• Randomised, double-blind, parallel group and multicentre • Treatments (12 week) • BFF 160/9.6µg BID MDI • BD 160µg BID MDI • Multi-country	• 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: 2024

Appendix: Glossary.

Daliresp/Daxas (oral PDE4 inhibitor)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase IV **PASS (post launch)** **NCT03381573**	COPD	124080	• A retrospective cohort trial comparing COPD patients aged 40 years and older with new exposure to roflumilast with up to 5 unexposed (i.e., not roflumilast-exposed) COPD controls matched by propensity score, age, sex and year of cohort entry • US, Germany, Sweden and Norway (using electronic healthcare databases)	• Primary endpoint: all-cause mortality (up to 5 years)	• FPCD: Q1 2017 • LPCD: Q4 2022 • Data readout: Q4 2022

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 • Data anticipated: 2024

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: 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: H2 2023

Appendix: Glossary.

Fasenra (IL-5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb **PONENTE** **NCT03557307**	Severe eosinophilic asthmatics receiving HD ICS + LABA and chronic OCS with or without additional asthma controller(s); age 18 years and older	598	• Arm 1: *Fasenra* 30mg Q8W s.c. • 38-week trial • Global trial – 16 countries	• Primary endpoint: reduction of oral corticosteroid dose	• FPCD: Q3 2018 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
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

Appendix: Glossary.

Fasenra (IL-5R mAb)

Severe, uncontrolled asthma, COPD and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
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: annualized rate of moderate or severe exacerbations over 56 weeks	• FPCD: Q4 2019 • Data anticipated: >2024

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

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 DESTINATION NCT03706079 Partnered (AMGEN)	Severe asthma; age 12 to 80 years	951	• Extension trial to NAVIGATOR and SOURCE • Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52-week trial (subjects from NAVIGATOR); 56-week trial (subjects from SOURCE) • Global trial – 18 countries	• Primary endpoint: exposure adjusted rates of AEs/SAEs • Secondary endpoints: annual asthma exacerbation rate	• FPCD: Q1 2019 • LPCD: Q4 2020 • Data readout: Q3 2022 • Primary endpoint met
Phase III PATH-HOME NCT03968978 Partnered (AMGEN)	Severe asthma; age 12 to 80 years	216	• Arm 1: *Tezspire* s.c. via AI • Arm 2: *Tezspire* s.c. via APFS • 24-week trial • Global trial – 4 countries	• Primary endpoint: proportion of health care professionals and patients/caregivers who successfully administrated Tezspire in clinic and at home with an APFS or an AI, respectively	• FPCD: Q2 2019 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III NOZOMI NCT04048343 Partnered (AMGEN)	Severe asthma; age 12 to 80 years	65	• Arm 1: *Tezspire* s.c. • 52-week trial • Japan only	• Primary endpoint: number of patients with AEs	• FPCD: Q2 2019 • LPCD: Q1 2020 • Data readout: Q2 2021 • Primary endpoint met
Phase II CASCADE NCT03688074 Partnered (AMGEN)	Severe asthma; age 18 to 75 years	116	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 28-week trial • Global trial – 5 countries	• Primary endpoint: number of airway submucosal inflammatory cells/mm2 of bronchoscopic biopsies	• FPCD: Q4 2018 • LPCD: Q4 2019 • Data readout: Q2 2021 • Primary endpoint met

Appendix: Glossary.

Tezspire (TSLP mAb)

Severe, uncontrolled asthma, COPD, CRSwNP & EoE

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DIRECTION** **NCT03927157** **Partnered (AMGEN)**	Severe asthma; age 18 to 80 years	396	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52week trial • Regional Asia trial – 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: 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
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: 2024
Phase III **CROSSING** **NCT05583227** **Partnered (AMGEN)**	Adult and pediatric 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: 2024

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, multicentre, 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 • Multi-country	• 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, multicentre and parallel-group • Treatments: 12-week treatment period) • BDA MDI 80/180µg QID • BDA MDI 160/180µg QID • BD MDI 160 µgQID • AS MDI 180 µgQID • placebo MDI QID • Multi-country	• 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, multicentre 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

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 study 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 • Data anticipated: >2024
Phase IIb/III INTREPID NCT03759288	Crohn's disease	928	• A 52-week, multicentre, randomised, double-blind, placebo- and active-controlled, operationally seamless Phase IIb/III, parallel group study 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 • Data anticipated: >2024
Phase II EXPEDITION NCT03616821	Ulcerative colitis	256	• A 54-week, multicentre, randomised, double-blind, placebo-controlled, parallel-group study 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 • Data anticipated: >2024

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 study 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 • Data anticipated: >2024
Phase I NCT05033431	Healthy volunteers	48	• Open-label study 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 • Data anticipated: H1 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	2352	• Randomised, double-blind, placebo-controlled, parallel group • Arm 1: tozorakimab dose i.v. + SoC • Arm 2: placebo i.v. + SoC • Global trial — 38 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

Appendix: Glossary.

tozorakimab (IL-33 ligand mAb)

Atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04212169**	Adults with atopic dermatitis	148	• Randomised, double-blind, placebo-controlled trial • Arm 1: tozorakimab s.c. • Arm 2: tozorakimab s.c. • Arm 3: tozorakimab s.c. • Arm 4: placebo s.c. • Global trial — 6 countries	• Primary endpoint: change from baseline at Week 16 in EASI score • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2019 • LPCD: Q2 2022 • Data readout: Q4 2022
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. • 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 anticipated: H1 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

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: annualized rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualized 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: annualized rate of moderate to severe COPD exacerbations (former smokers) • Secondary endpoints: annualized 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 study • 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

Appendix: Glossary.

Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III PROVENT NCT04625725	Adults with increased risk for inadequate response to active immunisation or having increased risk for SARS-CoV-2 infection	5197	• Double-blind, randomised, placebo-controlled, multicentre study to determine safety and efficacy in pre-exposure prophylaxis • Arm 1: *Evusheld* • Arm 2: placebo • *Evusheld*/placebo (2:1) • US, UK, Belgium, France and Spain	• Primary endpoint: positive symptomatic illness post-dose • Secondary endpoints: incidence of nucleocapsid antibodies, emergency visits, PCR positive, ADA to Evusheld in serum and Evusheld serum concentration	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q3 2021 • Primary endpoint met
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: 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

Appendix: Glossary.

Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
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

Appendix: Glossary.

Vaxzevria (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NCT04516746**	Healthy adults; age 18 to 65 years	32429	• Adaptive, double-blind, randomised placebo-controlled trial • *Vaxzevria* vs. placebo • US, Peru and Chile	• Primary endpoints: efficacy, safety, tolerability and reactogenicity • Secondary endpoint: immunogenicity	• FPCD: Q3 2020 • LPCD: Q1 2021 • Primary endpoint met

Appendix: Glossary.

AZD3152 (SARS-CoV-2 LAAB)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III **SUPERNOVA** **NCT05648110**	PhaseI: healthy adults; age 18 to 55 years Phase III: 12 years of age or older with conditions causing immune impairment, who are less likely to an adequate protective immune response after vaccination and are at high-risk of developing severe COVID-19	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 *Evusheld* 600mg administered i.m. in the anterolateral thigh on Day 1; participants will receive a second dose of their original randomised study intervention 6 months after Visit 1; main cohort randomisation will be stratified by SARS-CoV-2 vaccination status within 6 months prior to randomisation, prior SARS-CoV-2 infection within 6 months prior to randomisation, and *Evusheld* use within 12 months prior to randomisation; the duration of a main cohort participant's involvement in the study will be approximately 15 months from when the first dose of study intervention is administered • Global trial	• Primary endpoints: to evaluate the safety of AZD3152 and Evusheld and to compare the nAb responses to the SARS-CoV-2 Alpha variant in serum following AZD3152 and Evusheld administration • Secondary endpoints: to compare the efficacy of AZD3152 to Evusheld in the prevention of symptomatic COVID-19, to compare the nAb responses to the SARS-CoV-2 Omicron variant variants (BA.2 and/or BA.4/5 4/5) and the emerging dominant variant of concern circulating during the course of the study, to describe the incidence of symptomatic COVID -19, 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

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 • Data anticipated: H1 2023
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

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 • Data anticipated: H2 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

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	40	• MAD dose escalation in 3 cohorts with optional additional 2 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 study participants	• FPCD: Q2 2022 • Data anticipated: H2 2023

Appendix: Glossary.

AZD2693 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
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	72	• 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: 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

Appendix: Glossary.

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04630067**	Healthy volunteers (SAD) Heart failure (MAD)	104	• Multicentre SAD and MAD study • 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

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 anticipated: H1 2023

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

Appendix: Glossary.

AZD7503 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05143905**	Healthy volunteers	56	• SAD. 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: H1 2023

Appendix: Glossary.

balcinrenone/dapagliflozin (MR modulator + SGLT2i)

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: 2024

Appendix: Glossary.

cotadutide (GLP-1-glucagon agonist)

Diabetes/CKD, NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II/III PROXYMO-ADV NCT05364931	Patients with F2/F3 biopsy confirmed NASH	45	• Phase IIb/III randomised, double-blind, placebo-controlled	• Primary endpoints: proportion of participants with resolution of NASH • without worsening of liver fibrosis based on biopsy at • Week 48; proportion of participants with resolution of NASH • without worsening of liver fibrosis based on biopsy • at Week 84; proportion of participants with improvement of liver • fibrosis by at least one stage without worsening of • NASH based on biopsy at Week 84	• FPCD: Q4 2022 • Trial discontinued due to strategic portfolio prioritisation

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: high MEDI6570 dose • Arm 2: medium MEDI6570 dose • Arm 3: low MEDI6570 dose • Arm 4: placebo • 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: 2024

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

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 study • 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 anticipated: H1 2023

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: 2024

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 (n=166) • Arm 2: zibotentan dose B + *Farxiga* 10mg QD (n=83) • Arm 3: *Farxiga* 10mg + placebo QD (n=166) • 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

Appendix: Glossary.

atuliflapon (FLAP inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **FLASH** **NCT05251259**	Patients with moderate-to-severe uncontrolled asthma	1928	• Randomised, placebo-controlled, double-blind, multicentre, 2-part study with an active comparator (montelukast) arm, and 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: montelukast • Arm 5: placebo • Global trial	• Primary endpoint: time to first CompEx Asthma event	• FPCD: Q2 2022 • Data anticipated: 2024

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: H1 2023

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 anticipated: H1 2023
Phase I **NCT05630677**	Healthy volunteers	18	• BA study 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 anticipated: H1 2023

Appendix: Glossary.

AZD6793 (IRAK4)

Inflammatory diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05662033	Healthy volunteers	133	• Single blind, randomised, placebo-controlled study 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: H2 2023

Appendix: Glossary.

AZD7798 (humanized monoclonal antibody)

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

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

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, multicentre, 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 • 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 • Data anticipated: 2024

Appendix: Glossary.

mitiperstat (MPO inhibitor)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **CRESCENDO** **NCT05492877**	Moderate to severe COPD; age 40 to 80	288	• Randomised, double-blind study • 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: 2024

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 study	• Primary endpoint: change in respiratory parameters	• FPCD: Q3 2022 • LPCD: Q2 2023 • Data anticipated: H2 2023

Appendix: Glossary.

MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain

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 • Data readout: Q2 2022
Phase I **NCT05714254**	Healthy volunteers	48	• Randomised, double-blind, placebo-controlled MAD study • 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: 2024

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 study • 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

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

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: 2024
Phase I NCT04770428	Healthy volunteers (Japanese and Caucasian)	20	• MAD trial • Arm 1: MEDI7352 s.c. • Arm 2: placebo s.c. • Europe only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK and PD parameters, ADA	• FPCD: Q2 2021 • LPCD: Q3 2021 • Data readout: Q4 2021

Appendix: Glossary.

Rare Disease: approved medicines and late-stage pipeline

Koselugo (selumetinib, MEK inhibitor)

Neurofibromatosis Type 1 (NF1)

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	• Multicentre, international study with a parallel, randomised, double-blind, placebo-controlled, 2 arm design • Arm A: *Koselugo* 25mg/m2 BID • Arm B: 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: 2024

Appendix: Glossary.

Koselugo (selumetinib, MEK inhibitor)

Paediatric neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase II SPRINT NCT01362803 Partnered (NCI)	Paediatric NF1	75	• Single arm: *Koselugo* 25mg/m2 BID with 2 strata • Stratum 1: PN-related morbidity present at enrolment • Stratum 2: no PN-related morbidity present at enrolment	• Primary endpoints: complete partial and complete response rate measured by volumetric MRI, duration of response and functional outcomes/QoL	• FPCD: Q3 2015 • LPCD: Q4 2016 • Data readout: Q1 2019 • Primary endpoint met
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 study 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: 2024
Phase I Japan PK/Safety NCT04495127	Paediatric inoperable NF1-PN patients	12	• Open-label trial • *Koselugo* in Japanese paediatric NF1-PN patients	• Primary endpoint: safety • Secondary endpoints: PK parameters, anti-tumour effect	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q4 2021
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: H1 2023

Appendix: Glossary.

Ultomiris (anti-C5 mAb)

Haematology & nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1210-PNH-303** **NCT03748823**	PNH and aHUS	136	• *Ultomiris* s.c.	• Primary endpoint: Day 71 serum *Ultomiris* Ctrough	• FPCD: Q1 2019 • Data readout: Q2 2020 • Primary endpoint met
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** **NCT05746559**	CSA-AKI	736	• Randomised, double-blind, placebo-controlled, muticentre • *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: Q2 2023 • Data anticipated: >2024
Phase II **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: 2024

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 III **ALXN1210-MG-306** **NCT03920293**	Generalised myasthenia gravis	175	• Arm 1: *Ultomiris* • Arm 2: placebo	• Primary endpoint: change from baseline in MG-ADL total score at Week 26	• Data readout: Q2 2021 • 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: 2024
Phase II/III **ALXN1210-NMO-317** **NCT05346354**	Neuromyelitis optica spectrum disorder	12	• Arm 1: *Ultomiris* Q8W	• Primary endpoint: change from baseline in annualized relapse rate at Week 50	• FPCD: Q3 2022 • Data anticipated: >2024

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 sixallminute walk test, cause mortality and cardiovascular related hospitalisation over a 30-month period	• FPCD: Q4 2020 • Data anticipated: 2024

Appendix: Glossary.

ALXN1840 (bis-choline tetrathiomolybdate)

Wilson disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III WTX101-301 FoCus NCT03403205	Wilson disease	215	• Arm 1: ALXN1840 administered orally for 48 weeks at doses ranging from 15mg QOD up to a titrated dose of 60mg daily • Arm 2: SoC	• Primary endpoint: daily mean AUEC of dNCC • Secondary endpoint: change from baseline in the UWDRS Part II total score	• Data readout: Q3 2021 • Primary endpoint met
Phase II ALXN1840-WD-204 NCT04573309	Wilson disease	9	• Arm 1: participants administered ALXN1840 at a dose of 15mg QOD on Day 1 through Day 28 and then increased to 30mg/day on Day 29 through Day 39	• Primary endpoint: mean daily copper balance	• FPCD: Q3 2020 • Data readout: Q4 2022 • Primary endpoint not met
Phase II ALXN1840-WD-205 NCT04422431	Wilson disease	31	• Arm 1: ALXN1840 participants initiated at 15mg QD, then increased to 30mg QD at Week 6	• Primary endpoint: change from baseline at Week 48 in liver copper concentration	• FPCD: Q4 2020 • Data readout: Q4 2022 • Primary endpoint not met

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 sixminute 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 sixminute walk test and quality of life measures	• FPCD: Q1 2021 • Data anticipated: 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: H2 2023

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

Appendix: Glossary.

gefurulimab (ALXN1720, anti-C5 bi-specific V_HH antibody)

Neurology and nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN1720-MG-301** **NCT05556096**	Generalized 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: H1 2023

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

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 anticipated: H1 2023

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: 2024

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

Appendix: Glossary.

tarperprumig (ALXN1820, anti-properdin)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
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

Appendix: Glossary.

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**	Generalized 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: 2024

Appendix: Glossary.

List of abbreviations

14C	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 antibody
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
AE	Adverse event
aHUS	Atypical haemolytic uraemic syndrome
AI	Auto-injector
AI	Aromatase inhibitor
AKT	Protein kinase B
ALK	Anaplastic large-cell lymphoma kinase
ALL	Acute lymphocytic leukaemia
ALSFRS-R	Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised
AML	Acute myeloid leukaemia
APFS	Accessorised pre-filled syringe
APOL1	Apolipoprotein L1
AQLQ	Asthma quality of life questionnaire
AS	Albuterol sulfate
ASO	Antistreptolysin O
ATR	Ataxia telangiectasia and Rad3-related protein
ATTR-CM	Transthyretin amyloid cardiomyopathy
ATTRv-PN	Hereditary transthyretin-mediated amyloid polyneuropathy
AUC	Area under curve
AUCinf	Area under plasma concentration time curve from zero to infinity
AUClast	Area under plasma concentration curve from zero to the last quantifiable concentration
AUCt	Area under concentration-time curve
AUEC	Area under the effect-time curve
Avb8	Alpha v beta 8
B7-H4	B7 homolog 4
BA	Bioavailability
BAFF	B-cell activating factor
BCG	Bacillus Calmette-Guérin
BCL2	B-cell leukemia/lymphoma 2 protein
BCMA	B-cell maturation antigen
BCMA	B-cell maturation antigen
BDA	Budesonide albuterol
BFF	Budesonide and formoterol fumarate
BGF	Budesonide, glycopyrronium and formoterol fumarate
BICLA	British Isles Lupus Assessment Group-based Composite Lupus Assessment
BICR	Blinded independent central review
BID	Twice per day
BIG	Big Ten Cancer Research Consortium
BM	Biomarker
BMD	Bone mineral density
BMFI	Bone metastasis-free interval
BMI	Body mass index
BR	Bendamustine and rituximab
BRCAm	BReast CAncer gene-mutated
BRCAwt	BReast CAncer wild-type gene
BRD4	Bromodomain-containing protein 4
BTC	Biliary tract carcinoma
BTK	Bruton's tyrosine kinase
BVAS	Birmingham Vasculitis Activity Score
C5	Complement component 5
CA-125	Cancer antigen-125
CAD	Coronary artery disease
CAGR	Compound annual growth rate
CBP	Cardiopulmonary bypass
CBR	Clinical benefit rate
CD	Cluster of differentiation
CD8	Cluster of differentiation 8
CDAI	Clinical Disease Activity Index
CDK	Cyclin-dependent kinase
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
cHL	Classic Hodgkin lymphoma
CLD	Chronic lung disease
CLL	Chronic lymphocytic leukaemia
CMAX	Maximum observed plasma concentration
cMET	Tyrosine-protein kinase mesenchymal epithelial transition factor
CMML	Chronic myelomonocytic leukaemia
CNS	Central nervous system
CompEx	Composite endpoint for exacerbations
COPD	Chronic obstructive pulmonary disease
CPI	Checkpoint inhibitor
cPR	Central pathological review
CR	Complete response
CRC	Colorectal cancer
CrCl	Creatinine clearance
CRR	Complete response rate
CRR	Complete renal response
CSA-AKI	Cardiac surgery-associated acute kidney injury
CTC	Circulating tumour cell
CTCAE	Common Terminology Criteria for Adverse Events
ctDNA	Circulating tumor DNA
CTLA-4	Cytotoxic T-lymphocyte-associated antigen-4
CTx	Chemotherapy
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CXCR2	C-X-C Motif chemokine receptor 2
CyBorD	Cyclophosphamide, bortezomib and dexamethasone
Dato-DXd	Datopotamab deruxtecan
DCR	Disease control rate
DDFS	Distant disease-free survival
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
dNCC	Directly measured non-ceruloplasmin-bound copper
DoCR	Durability of complete response
DoR	Duration of response
DPI	Dry powder inhaler
DRFI	Disease recurrence-free interval
DSQ	Dysphagia Symptom Questionnaire
DXA	Dual energy X-ray absorptiometry
EBRT	External beam radiation therapy

List of abbreviations

ECG	Electrocardiogram
EFS	Event-free survival
EG	Eosinophilic gastritis
EGE	Eosinophilic gastroenteritis
eGFR	Estimated glomerular filtration rate
EGFRm	Epidermal growth factor receptor-mutated
EGPA	Eosinophilic granulomatosis with polyangiitis
EoE	Eosinophilic oesophagitis
ER	Oestrogen receptor
ERK	Extracellular signal-regulated kinase
E-RS:COPD	Evaluating Respiratory Symptoms in Chronic Obstructive Pulmonary Disease
ESAI	Eczema Area and Severity Index
ESCC	Esophageal squamous cell carcinoma
ESR1	Oestrogen receptor 1
ESRD	End-stage renal disease
ET	Endocrine therapy
EVH	Extravascular haemolysis
FAF	Fundus autofluorescence
FCR	Fludarabine, cyclophosphamide and rituximab
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FEV1	Forced expiratory volume in 1 second
FGFR	Fibroblast growth factor receptor
FL	Follicular lymphoma
FOLFOX	Folinic acid, fluorouracil and oxaliplatin
FOXP3	Forkhead box P3
FPCD	First patient commenced dosing
FPG	Fasting plasma glucose
GA	Geographic atrophy
GBM	Glioblastoma
gBRCAm	Germline BRCA-mutated
GC	Gastric cancer
GCB	Germinal center B-cell
GEJ	Gastric/gastroesophogeal junction
GEJC	Gastroesophageal junction cancer
GFF	Glycopyrronium and formoterol fumarate
GI	Gastrointestinal
GLP-1	Glucagon-like peptide-1
GMFR	Geometric mean fold rise
gMG	Generalised myasthenia gravis
GMT	Geometric mean titer
H1	H1-antihistamine
hADME	Human mass balance
HCC	Hepatocellular carcinoma
HD	High dose
HDL-C	High-density lipoprotein cholesterol
HER2+	Human epidermal growth factor receptor 2-positive
HER2-low	Human epidermal growth factor receptor 2-low
HER2-neg	Human epidermal growth factor receptor 2-negative
HES	Hyper eosinophilic syndrome
HF	Heart failure
HFpEF	Heart failure with preserved ejection fraction
HFrEF	Heart failure with reduced ejection fraction
HGFR	Met/hepatocyte growth factor receptor
HGSC	High-grade serous carcinoma
hHF	Hospitalisation for heart failure
HIF-PHI	Hypoxia inducible factor-prolyl hydroxylase inhibitor
HNSCC	Head and neck squamous-cell carcinoma
HPD	Hyperprogressive disease
HPF	High-power field
HPP	Hypophosphatasia
HR+	Hormone receptor-positive
HRD	Homologous recombination deficiency
HRD+	Homologous recombination deficiency-positive
HRRm	Homologous recombination repair-mutated
HVPG	Hepatic venous pressure gradient
i	Inhibitor
i.m.	Intramuscular
i.v.	Intravenous
IA	Investigator-assessed
ICR	Independent central review
ICS	Inhaled corticosteroid
ICU	Intensive care unit
IDFS	Invasive disease-free survival
IgAN	Immunoglobulin A nephropathy
IHF	Impaired hepatic function
IL	Interleukin
IL-12	Interleukin-12
IL-33	Interleukin-33
IL-5R	Interleukin-5 receptor
IMAC-TIS	International Myositis Assessment And Clinical Studies-Total Improvement Score
INV	Investigator review
IO	Immuno-oncology
IPFS	Invasive progression-free survival
IRAK4	Interleukin-1 receptor-associated kinase 4
IRC	Independent review committee
ISS	Investigator-sponsored studies
ISS7	Itch-severity score (weekly)
LAAB	Long-acting antibody
LABA	Long-acting beta agonist
LAMA	Long-acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LDH	Lactate dehydrogenase
LICA	Ligand-conjugated ASO
LN	Lupus nephritis
LOS	Length of stay
LPCD	Last patient commenced dosing
LSD	Last subject dosed
LV	Left ventricle
m	Mutation
mAb	Monoclonal antibody
MABA	Muscarinic antagonist-beta2 agonist
MACE	Major adverse cardiac events
MAD	Multiple ascending dose
MAKE	Major adverse kidney events
MCC	Mucociliary clearance
MCL	Mantle cell lymphona
mCRPC	Metastatic castrate-resistant prostate cancer
MDI	Metered-dose inhaler
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Mesenchymal epithelial transition factor
MG-ADL	Myasthenia Gravis-Activities of Daily Living
MI	Myocardial infarction

List of abbreviations

MMT	Mixed meal test
mPFS	Median progression-free survival
MPO	Myeloperoxidase
mPR	Major pathological response
MRI	Magnetic resonance imaging
mRNA	Messenger ribonucleic acid
MSA	Multiple system atrophy
MTD	Maximum tolerated dose
mTNBC	Metastatic triple-negative breast cancer
MZL	Marginal zone lymphoma
nAb	Neutralising antibody
NaC	Sodium channel
NASH	Non-alcoholic fatty liver disease
NCFB	Non-cystic fibrosis bronchiectasis
NCI	National Cancer Institute
NCPV	Noncalcified plaque volume
NF1	Neurofibromatosis type 1
NF1-PN	Neurofibromatosis type 1 with plexiform neurofibromas
ng	Next-generation
NGF	Nerve growth factor
NHA	New hormonal agents
NHL	Non-Hodgkin's lymphoma
NIH	National Institute of Health
NKTCL	Extranodal natural killer T-cell lymphoma
NMOSD	Neuromyelitis optica spectrum disorder
NRG	National Clinical Trials Network in Oncology
NSCLC	Non-small cell lung cancer
OBD	Optimal biological dose
OCS	Oral corticosteroid
OD	Once daily
OGTT	Oral glucose tolerance test
OR	Objective response
ORR	Overall response rate
OS	Overall surival
PALB2m	Partner and localizer of BRCA2-mutated
PARP-1sel	Poly ADP ribose polymerase-1 selective
PASI	Psoriasis area severity index
PBD	Pyrrolobenzodiazepine
PCD	Plasma cell dyscrasia
pCR	Pathological complete response
PCSK9	Proprotein convertase subtilisin/kexin type 9
PD	Pharmacodynamics
PD-1	Programmed cell death protein-1
PDAC	Pancreatic ductal adenocarcinoma
PDE4	Phosphodiesterase type 4
PD-L1	Programmed death-ligand 1
Peak	Maximum
PET	Positron-emission tomography
PFS	Progression-free survival
PFS2	Time to second disease progression or death
PgR	Progesterone receptor
PI3K	Phosphoinositide 3 kinase
PIK3CA	Phosphatidylinositol 3 kinase catalytic alpha gene
PK	Pharmacokinetic
PLL	Prolymphocytic leukaemia
pMDI	Pressurised metered-dose inhaler
PN	Plexiform neurofibroma
PNH	Paroxysmal nocturnal haemoglobinuria
POC	Proof of concept
POM	Proof of mechanism
post-BD	Post-bronchodilator
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
PSR	Platinum-sensitive relapsed
PTCL	Peripheral T-cell lymphoma
PTEN	Phosphatase and tensin homolog gene
Q1W	Every one week
Q4W	Every four weeks
Q8W	Every eight weeks
QD	Once daily
QID	Four times per day
QOD	Evey other day
QoL	Quality of life
QTcF	Corrected QT interval by Fredericia
R/R	Relapsed/refractory
RA	Rheumatoid arthritis
RAAS	Renin-angiotensin-aldosterone system
RECIST	Response Evaluation Criteria in Solid Tumours
REiNS	Response Evaluation in Neurofibromatosis and Schwannomatosis
RFS	Relapse-free survival
rhLCAT	Recombinant human lecithin-cholesterol acyltransferase
rNDV	Recombinant Newcastle disease virus
RORγ	Related orphan receptor gamma
RP2D	Recommended Phase II dose
rPFS	Radiographic progression-free survival
RR	Response rate
RT	Radiation therapy
s.c.	Subcutaneous
SABA	Short-acting beta2-agonist
SAD	Single ascending dose
SAE	Serious adverse event
SBRT	Stereotactic body radiation therapy
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
siRNA	Small interfering ribonucleic acid
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
SS	Steady state
STAT3	Signal transducer and activator of transcription 3
sUA	Serum uric acid
T790M	Threonine 790 substitution with methionine

List of abbreviations

TACE	Transarterial chemoembolization
tBRCAm	Tumour (somatic) BRCA-mutated
TEAE	Treatment-emergent adverse event
TESAE	Treatment-emergent serious adverse event
TFST	Time to first subsequent therapy or death
THP	Paclitaxel, trastuzumab and pertuzumab
TID	Three times per day
TIGIT	T-cell immunoreceptor with Ig and ITIM domains
TIM3	T-cell immunoglobulin and mucin domain 3
TJC	Tender joint count
TKI	Tyrosine kinase Inhibitor
TLR	Toll-like receptor 9
TMA	Thrombotic microangiopathy
Tmax	Time to reach maximum observed plasma concentration
TNF	Tumour necrosis factor
TPS	Tumour proportion score
TSLP	Thymic stromal lymphopoietin
TTD	Time to treatment discontinuation
TTF	Time to treatment failure
TTNT	Time to next therapy
TTP	Time to tumour progression
TTR	Time to treatment response
UACR	Urine albumin creatinine ratio
u-LTE4	Urinary leukotriene E4
UMEC	Umeclidinium
URAT1	Uric acid transporter 1
UWDRS	Unified Wilson Disease Rating Scale
VEGF	Vascular endothelial growth factor
VHH	Single domain antibody