Clinical trials appendix

Q3 2022 results update

Pipeline catalysts for 2022 - 2023

Oncology **BioPharmaceuticals** **Rare Disease**

	H2 2022	H1 2023	H2 2023
Regulatory decision	***Imfinzi*** – biliary tract cancer (TOPAZ-1) (JP) ***Imfinzi*** – liver cancer (1L) (HIMALAYA) (JP) ***Imfinzi*** – NSCLC (1L) (POSEIDON) ***Lynparza*** – prostate cancer (1L) (PROpel) ***Enhertu*** – HER2+ breast cancer (2L) (DESTINY-Breast03) (JP) ***Calquence*** – CLL (ELEVATE-TN) (JP)	***Imfinzi*** – biliary tract cancer (TOPAZ-1) (EU) ***Imfinzi*** – liver cancer (1L) (HIMALAYA) (EU) ***Lynparza*** – breast cancer (OlympiAD) (CN) ***Enhertu*** – HER2-low breast cancer (3L) (DESTINY-Breast04) (EU) ***Enhertu*** – HER2+ gastric cancer (2L) (DESTINY-Gastric01) (EU) ***Enhertu*** – HER2+ breast cancer (2L) (DESTINY-Breast03) (CN) ***Enhertu*** – HER2-low breast cancer (3L) (DESTINY-Breast04) (JP, CN) ***Farxiga*** – HFpEF (DELIVER) **PT027** – asthma (MANDALA/DENALI) (US) ***Ultomiris*** – NMOSD (CHAMPION-NMOSD)	***Soliris*** – gMG (CN) ***Koselugo*** – NF1-PN (SPRINT) (CN)
Regulatory submission and/or acceptance	***Calquence*** – CLL (ASCEND) CN) **eplontersen** – ATTRv-PN (NEURO-TTRansform) (US) ***Beyfortus*** – RSV (MELODY/MEDLEY) (US) ***Evusheld*** – COVID-19 (TACKLE/PROVENT) (CN)	***Imfinzi*** – liver cancer (locoregional) (EMERALD-1) ***Imfinzi*** – liver cancer (adjuvant) (EMERALD-2) ***Imfinzi*** – NSCLC (1L) (PEARL) ***Lynparza*** – gBRCA breast cancer (adjuvant) (OlympiA) (CN) **capivasertib** – HR+/HER2-neg breast cancer (1L) (CAPItello-291) **Dato-DXd** – NSCLC (3L) (TROPION-Lung01) ***Beyfortus*** – RSV (MELODY/MEDLEY) (JP, CN) **danicopan** – PNH with extravascular haemolysis	***Tagrisso*** – EGFRm NSCLC (1L) (FLAURA2) ***Tagrisso*** – EGFRm NSCLC (unresectable Stg. III) (LAURA) ***Imfinzi*** – biliary tract cancer (TOPAZ-1) (CN) ***Imfinzi*** – NSCLC (unresectable, Stg. III) (PACIFIC-2) ***Imfinzi*** – bladder cancer (muscle invasive) (NIAGARA) ***Imfinzi*** – bladder cancer (1L) (NILE) ***Imfinzi*** – NSCLC (neoadjuvant) (AEGEAN) ***Imfinzi*** – SCLC (limited-stage) (ADRIATIC) **capivasertib** – TNBC (locally adv./met.) (CAPItello-290)
Key Phase III data readouts	***Imfinzi*** – liver cancer (locoregional) (EMERALD-1) ***Imfinzi*** – NSCLC (1L) (PEARL)	***Tagrisso*** – EGFRm NSCLC (1L) (FLAURA2) ***Imfinzi*** – SCLC (limited-stage) (ADRIATIC) ***Imfinzi*** – NSCLC (neoadjuvant) (AEGEAN) ***Imfinzi*** – liver cancer (adjuvant) (EMERALD-2) ***Lynparza*** – ovarian cancer (1L) (DUO-O) **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*** – bladder cancer (muscle invasive) (NIAGARA) ***Imfinzi*** – bladder cancer (1L) (NILE) ***Lynparza*** – endometrial cancer (1L) (DUO-E) ***Enhertu*** – HER2-low breast cancer (2L) (DESTINY-Breast06) ***Calquence*** – MCL (1L) (ECHO) **capivasertib** – TNBC (locally adv./met.) (CAPItello-290) ***Farxiga*** – myocardial infarction (DAPA-MI) ***Fasenra*** – EGPA (MANDARA) ***Fasenra*** – HES (NATRON)

1L = 1st-line; 2L = 2nd-line; 3L = 3rd-line; NSCLC = non-small cell lung cancer; HER2+ = human epidermal growth factor receptor 2-positive; HER2-low = human epidermal growth factor receptor 2-low; CLL = chronic lymphocytic leukaemia; HFpEF = heart failure with preserved ejection faction; RSV = respiratory syncytial virus; CKD = chronic kidney disease; ATTRv-PN = hereditary transthyretin-mediated amyloid polyneuropathy; NMOSD = neuromyelitis optica spectrum disorder; gBRCAm = germline BRCA mutated; HR+ = hormone receptor-positive; HER2-neg = human epidermal growth factor receptor 2 negative; Dato-DXd = datopotamab deruxtecan; EoE = eosinophilic oesophagitis; PNH = paroxysmal nocturnal haemoglobinuria; gMG = generalised myasthenia gravis; NF1= neurofibromatosis type 1; PN = plexiform neurofibromas; EGFRm = epidermal growth factor receptor mutated; SCLC = small cell lung cancer; TNBC = triple negative breast cancer; MCL = mantle cell lymphoma; EGPA = eosinophilic granulomatosis with polyangiitis; HES = hyper eosinophilic syndrome.

CTA 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)
Pipeline: Next wave	tozorakimab (IL-33) ngCOVID-19 LAAB eplontersen (LICA) mitiperstat (MPO) cotadutide (GLP-1/Glucagon)	Dato-DXd (TROP2 ADC) volrustomig (PD1-CTLA4) capivasertib (AKT) camizestrant (ngSERD) AZD2936 (PD1-TIGIT) AZD5305 (PARP-1sel)	vemircopan (oral Factor D) gefurulimab (C5 mini-body) ALXN1850 (ngHPP)

Movement since Q2 2022 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
NME **ALXN2030** siRNA targeting complement C3 nephrology **ALXN2080#** oral factor D inhibitor healthy volunteers **AZD6234** long-acting amylin obesity with related comorbidities **AZD7798** humanised monoclonal antibody targets T cells subset Crohn's disease **AZD9574** PARP inhibitor advanced solid malignancies	**NME** **AZD8205** B7-H4 targeting antibody drug conjugate solid tumours **Additional indication** **vemircopan (ALXN2050)** oral Factor D inhibitor proliferative lupus nephritis or immunoglobulin A nephropathy	**Life-cycle management** ***Tagrisso* + *Orpathys* SAFFRON#** EGFR inhibitor + MET inhibitor advanced EGFRm non-small cell lung cancer	**Life-cycle management** ***Farxiga/Forxiga* DELIVER[1]** SGLT-2 inhibitor worsening HF or CV death in patients with chronic heart failure (HFpEF) ***Ultomiris* CHAMPION-NMOSD[1]** anti-complement C5 mAb neuromyelitis optica spectrum disorder

Phase progressions based on first patient dose achievement.
[1] Submission accepted [2] Approved # Partnered and/or in collaboration ¶ Registrational Phase II/III trial

Movement since Q2 2022 update

Removed from Phase I	Removed from Phase II	Removed from Phase III	Removed from registration
NME **AZD5991** MCL1 inhibitor haematological malignancies **MEDI1191#** IL12 mRNA solid tumours **MEDI8367** Avb8 chronic kidney disease	**NME** **AZD8233** PCSK9-ASO hypercholesterolemia **Additional indications** **atuliflapon (AZD5718)** FLAP coronary artery disease / chronic kidney disease **cotadutide** GLP-1/glucagon dual agonist type-2 diabetes, obesity and diabetic kidney disease	**Additional indication** **monalizumab + cetuximab INTERLINK-1[#]** NKG2A mAb + EGFR mAb 2L+ relapsed metastatic head and neck squamous cell cancer **Life-cycle management** ***Fasenra* MAHALE** IL-5R mAb non-cystic fibrosis bronchiectasis **Fasenra MESSINA** IL-5R mAb eosinophilic esophagitis ***Imfinzi* + CTx MERMAID-1** PD-L1 mAb + CTx stage II-III adjuvant non-small cell lung cancer ***Imfinzi* MERMAID-2** PD-L1 mAb stage II-III premetastatic non-small cell lung cancer ***Ultomiris*** anti-complement C5 mAb complement-mediated thrombotic microangiopathy	**NME** ***Beyfortus* (nirsevimab)[#2]** RSV mAb-YTE passive RSV immunisation ***Imfinzi* + *Imjudo* (tremelimumab) HIMALAYA[#2]** PD-L1 mAb + CTLA-4 mAb 1st-line hepatocellular carcinoma **Life-cycle management** ***Enhertu* DESTINY-Lung01[#2]** HER2 targeting antibody drug conjugate HER2-over-expressing or -mutated, unresectable and/or metastatic non-small cell lung cancer ***Enhertu* DESTINY-Breast04[#2]** HER2 targeting antibody drug conjugate HER2-low, unresectable and/or metastatic breast cancer subjects ***Evusheld* TACKLE[2]** LAAB combination treatment of COVID-19 ***Imfinzi* + CTx TOPAZ-1[#2]** PD-L1 mAb + CTx 1st-line biliary tract cancer ***Ultomiris*[2]** anti-complement C5 mAb subcutaneous, paroxysmal nocturnal haemoglobinuria and atypical haemolytic uraemic syndrome

Phase progressions based on first patient dose achievement.
[1] Submission accepted [2] Approved [#] Partnered and/or in collaboration [¶] Registrational Phase II/III trial

Q3 2022 Oncology new molecular entity[1] pipeline

Phase 1

10 New Molecular Entities

- AZD0466#
  BCL2/xL haematological malignancies
- AZD1390
  ATM glioblastoma
- AZD2936 ARTEMIDE-1#
  PD1/TIGIT bispecific mAb solid tumours
- AZD7789
  PD1/TIM3 bispecific mAb solid tumours, haematological malignancies
- AZD8701 +/- *Imfinzi*#
  FOXP3 +/- PD-L1 solid tumours
- AZD8853
  GDF-15 solid tumours
- AZD9574
  PARP inhibitor advanced solid malignancies
- IPH5201#
  CD39 solid tumours
- volrustomig (MEDI5752) + lenvatinib
  PD-1/CTLA-4+VEGF advanced RCC
- MEDI9253
  rNDV IL12 solid tumours

Phase 2

11 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
- AZD8205
  B7-H4 targeting ADC solid tumours
- camizestrant
  SERD HR+ breast
- capivasertib
  AKT prostate cancer
- ceralasertib
  ATR solid tumours
- *Imfinzi* + monalizumab#
  PD-L1+NKG2A solid tumours
- volrustomig (MEDI5752)
  PD-1/CTLA-4 solid tumours
- oleclumab + CTx or *Imfinzi* + oleclumab + CTx
  CD73 + chemo or PD-L1 + CD73 + chemo metastatic pancreatic cancer

Phase 3

15 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
- datopotamab deruxtecan TROPION Lung08#
  TROP2 ADC 1L metastatic NSCLC
- datopotamab deruxtecan TROPION-Breast02#
  TROP2 ADC 1L TNBC
- *Imfinzi* + *Imjudo* (tremelimumab) + SoC NILE
  PD-L1+CTLA-4+SoC 1L urothelial cancer
- *Imfinzi* +/- *Imjudo* (tremelimumab) + CRT ADRIATIC#
  PD-L1+/-CTLA-4+CRT LS-SCLC
- camizestrant + palbociclib SERENA-4
  SERD+CDK4/6 1L HR+ HER2- breast cancer
- capivasertib + CTx CAPItello-290
  AKT+chemotherapy mTNBC 1L
- capivasertib + fulvestrant + palbociclib CAPItello-292
  AKT+fulvestrant+CDK4/6 1L triplet in early relapse/ET resistant locally advanced or mBC
- datopotamab deruxtecan TROPION-Breast01#
  TROP2 ADC 2-3L HR+ HER2- breast cancer
- datopotamab deruxtecan TROPION-Lung01#
  TROP2 ADC 2L+ NSCLC with or without actionable genomic alterations
- *Imfinzi* + *Imjudo* (treme) + TACE +/- lenvatinib EMERALD-3
  PD-L1+CTLA4+VEGF+/-chemo-embolization locoregional HCC
- *Imfinzi* +- oleclumab +- monalizumab PACIFIC-9#
  PD-L1+NKG2A or PD-L1+CD73 unresectable stage III NSCLC

Under review

1 New Molecular Entity

- *Imfinzi* +/- *Imjudo* (tremelimumab) + CTx POSEIDON
  PD-L1+/-CTLA-4+CTx 1L NSCLC

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/III trial

● Precision medicine approach being explored

Q3 2022 Oncology life-cycle management[1] pipeline

Phase 1

2 Projects

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

Phase 2

12 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) MAGELLAN# PD-L1+multiple novel ONC therapies+/-CTx 1L mNSCLC
- *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 3

32 Projects

- *Calquence* + R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL
- *Enhertu* DESTINY-Breast05# HER2 ADC HER2+ post-neoadjuvant high-risk breast cancer
- *Enhertu* DESTINY-Breast09# HER2 ADC HER2+ breast cancer 1L
- *Imfinzi* + CRT KUNLUN# PD-L1+CRT locally-advanced ESCC
- *Imfinzi* + CRT PACIFIC-5 (China)# PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi* + EV +/- *Imjudo* (tremelimumab) VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 MIBC
- *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* + Orpathys SAFFRON# EGFR + MET advanced EGFRm non-small cell lung cancer
- *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-Breast02# HER2 ADC HER2+ breast cancer
- *Enhertu* DESTINY-Breast11# HER2 ADC Neoadjuvant HER2+ breast cancer
- *Enhertu* DESTINY-Lung04# HER2 ADC HER2m NSCLC 1L
- *Imfinzi* + CTx neoadjuvant AEGEAN# PD-L1+CTx locally-advanced stage II-III NSCLC
- *Imfinzi* + domvanalimab + CTx PACIFIC-8# PD-L1+TIGIT+CTx unresectable stage III NSCLC
- *Imfinzi* + VEGF EMERALD-2# PD-L1+VEGF adjuvant HCC
- *Imfinzi* post-SBRT PACIFIC-4# PD-L1 mAb post-SBRT stage I/II non-small cell lung cancer
- *Lynparza* MONO-OLA1# PARP 1L BRCAwt ovarian cancer
- *Tagrisso* + CTx FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC
- *Tagrisso* LAURA EGFR inhibitor stage III EGFRm NSCLC
- *Calquence* ECHO# BTK inhibitor 1L MCL
- *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* + FLOT MATTERHORN# PD-L1+CTx Neo-adjuvant/adjuvant gastric cancer
- *Imfinzi* PEARL PD-L1 1L metastatic NSCLC
- *Lynparza* + *Imfinzi* + bevacizumab DUO-O# PARP+PD-L1+VEGF 1L ovarian cancer
- *Orpathys* + *Imfinzi* SAMETA# MET+PD-L1 1L papillary renal cell carcinoma
- *Tagrisso* +/- CTx neoadjuvant NeoADAURA EGFR+/-CTx stage II/III resectable EGFRm NSCLC

Under review

1 Project

- *Lynparza* + abiraterone PROpel# PARP+NHA prostate 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

● Precision medicine approach being explored

Q3 2022 BioPharmaceuticals new molecular entity[1] pipeline

Phase 1
16 New Molecular Entities

- AZD0780 PCSK9 dyslipidemia
- AZD2693 NASH resolution non-alcoholic steatohepatitis
- AZD3427 relaxin mimetic CV disease
- AZD4604 inhaled JAK1 asthma
- AZD5462 relaxin mimetic CV disease
- AZD7798 humanized monoclonal antibody targets T cells subset Crohn's disease
- MEDI0618 PAR2 antagonist osteoarthritis pain
- MEDI1814# amyloid beta mAb alzheimer's disease
- AZD2373 podocyte health nephropathy
- AZD3366 CD39L3 cardiovascular disease
- AZD4041# orexin 1 receptor antagonist opioid use disorder
- AZD5055 porcupine inhibitor idiopathic pulmonary fibrosis
- AZD6234 long-acting amylin obesity with related comorbidities
- AZD7503 ASO non-alcoholic steatohepatitis
- AZD8630# Inhaled TSLP FAb asthma
- MEDI1341# alpha synuclein mAb parkinson's disease

Phase 2
14 New Molecular Entities

- atuliflapon (AZD5718) FLAP asthma
- mitiperstat (AZD4831) MPO HFpEF
- brazikumab EXPEDITION IL-23 mAb ulcerative colitis
- MEDI6570 LOX-1 CV disease
- navafenterol# MABA COPD
- tozorakimab IL-33 COVID-19
- tozorakimab IL-33 atopic dermatitis
- AZD1402# inhaled IL-4Ra asthma
- balcinrenone (AZD9977)/dapagliflozin MR+SGLT2 heart failure with CKD
- cotadutide GLP-1/glucagon dual agonist NASH
- MEDI7352 NGF/TNF OA pain / PDN
- tozorakimab IL-33 diabetic kidney disease
- tozorakimab IL-33 asthma
- zibotentan + *Farxiga/Forxiga* endothelin A receptor antagonist + SGLT2 CKD

Phase 3
4 New Molecular Entities

- brazikumab INTREPID IL-23 Crohn's disease
- eplontersen# LICA hATTR-Polyneuropathy
- eplontersen# LICA ATTR-Cardiomyopathy
- tozorakimab IL-33 COPD

Under review
1 New Molecular Entity

- PT027# ICS/SABA asthma

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/III trial

● Precision medicine approach being explored

Q3 2022 BioPharmaceuticals life-cycle management[1] pipeline

Phase 1	Phase 2	Phase 3		Reg
0 Projects	4 Projects	14 Projects		1 Project
	Andexxa (ALXN2070) anti-factor Xa reversal urgent surgery	*Breztri*/Trixeo (PT010) KALOS LOGOS LABA/LAMA/ICS asthma	*Farxiga/Forxiga* DAPA-MI SGLT-2 prevention of HF and CV death following a myocardial infarction	*Farxiga/Forxiga* DELIVER SGLT-2 HFpEF
	Fasenra ARROYO IL-5R chronic spontaneous urticaria	*Fasenra* FJORD IL-5R bullous pemphigoid	*Fasenra* HUDSON IL-5R eosinophilic gastritis and eosinophilic gastroenteritis	
	roxadustat# HIFPH anaemia chemotherapy induced anaemia	*Fasenra* MANDARA IL-5R eosinophilic granulomatosis with polyangiitis	*Fasenra* NATRON IL-5R hypereosinophilic syndrome	
	Tezspire COURSE# TSLP chronic obstructive pulmonary disease	*Fasenra* ORCHID# IL-5R nasal polyps	*Fasenra* RESOLUTE# IL-5R chronic obstructive pulmonary disease	
		Lokelma DIALIZE-Outcomes potassium binder CV outcomes in patients on chronic haemodialysis with hyperkalaemia	*Lokelma* STABILIZE-CKD potassium binder hyperkalaemia in CKD	
		roxadustat# HIFPH anaemia MDS	*Saphnelo* TULIP-SC# Type I IFN receptor SLE SC	
		Saphnelo# Type I IFN receptor mAb lupus nephritis	*Tezspire* WAYPOINT# TSLP nasal polyps	

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

● Precision medicine approach being explored

Q3 2022 Rare Disease pipeline[1]

Phase 1 7 Projects	Phase 2 5 Projects	Phase 3 5 Projects	Reg 1 Project
ALXN1820 anti-properdin bi-specific haematology	danicopan (ALXN2040) factor D geographic atrophy	acoramidis (ALXN2060)# oral TTR stabilizer transthyretin amyloid cardiomyopathy	*Ultomiris* (ALXN1210) anti-complement C5 mAb neuromyelitis optica spectrum disorder
ALXN1850 next gen TNSALP ERT hypophosphatasia	*Ultomiris* (ALXN1210) anti-complement C5 mAb dermatomyositis	ALXN1840 bis-choline tetrathiomolybdate Wilson Disease	
ALXN1910 next gen TNSALP ERT bone metabolism	vemircopan (ALXN2050) oral Factor D proliferative lupus nephritis or immunoglobulin A nephropathy	CAEL-101 fibril-reactive mAb AL amyloidosis	
ALXN2030 siRNA targeting complement C3 nephrology	vemircopan (ALXN2050) oral factor D inhibitor paroxysmal nocturnal haemoglobinuria	danicopan (ALXN2040) factor D PNH with clinically relevant extravascular haemolysis	
ALXN2080 oral factor D healthy volunteers	vemircopan (ALXN2050) oral factor D inhibitor generalized myasthenia gravis	*Ultomiris* (ALXN1210) anti-complement haematopoietic stem cell transplant-associated thrombotic micorangiopathy	
gefurulimab (ALXN1720) humanised bispecific VHH antibody generalised myasthenia gravis			
NI006# TTR depleter transthyretin amyloid cardiomyopathy			

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

● Precision medicine approach being explored

Estimated key regulatory submission acceptances

	H2 2022	H1 2023	H2 2023	>2023		
NME	eplontersen hATTR-Polyneuropathy	danicopan (ALXN2040) PNH with clinically relevant extravascular haemolysis	*Imfinzi* +/- *Imjudo* (tremelimumab) + CRT ADRIATIC LS-SCLC	capivasertib + docetaxel CAPItello-280 mCRPC prostate cancer	brazikumab INTREPID Crohn's disease	
		datopotamab deruxtecan TROPION-Lung01 2L+ NSCLC with or without actionable genomic alterations	*Imfinzi* + *Imjudo* (tremelimumab) + SoC NILE 1L urothelial cancer	capivasertib + fulvestrant + palbociclib CAPItello-292 1L triplet in early relapse/ET resistant locally advanced or mBC	eplontersen ATTR-Cardiomyopathy	
		capivasertib + fulvestrant CAPItello-291 2L and beyond in AI resistant locally advanced or mBC	capivasertib + CTx CAPItello-290 mTNBC 1L	capivasertib + abiraterone CAPItello-281 PTEN deficient mHSPC	*Imfinzi* +- oleclumab +- monalizumab PACIFIC-9 unresectable stage III NSCLC	
				camizestrant + palbociclib SERENA-4 1L HR+ HER2- breast cancer	*Imfinzi* + *Imjudo* (treme) + TACE +/- lenvatinib EMERALD-3 locoregional HCC	ALXN1840 Wilson Disease
				camizestrant + CDK4/6i SERENA-6 1L HR+ HER2- ESR1m breast cancer	datopotamab deruxtecan TROPION Lung08 1L metastatic NSCLC	acoramidis (ALXN2060) transthyretin amyloid cardiomyopathy
				datopotamab deruxtecan TROPION-Breast01 2-3L HR+ HER2- breast cancer	datopotamab deruxtecan TROPION-Breast02 1L TNBC	CAEL-101 AL amyloidosis
LCM	*Imfinzi* + VEGF + TACE EMERALD-1 locoregional HCC	*Enhertu* DESTINY-Breast02 HER2+ breast cancer	*Imfinzi* + CRT PACIFIC-2 locally-advanced stage III NSCLC	*Calquence* + R-CHOP ESCALADE 1L DLBCL	*Imfinzi* POTOMAC non-muscle invasive bladder cancer	*Saphnelo* lupus nephritis
		Imfinzi PEARL 1L metastatic NSCLC	*Imfinzi* + CTx neoadjuvant AEGEAN locally-advanced stage II-III NSCLC	*Calquence* + venetoclax + obinutuzumab AMPLIFY 1L CLL	*Lynparza* + *Imfinzi* + bevacizumab DUO-O 1L ovarian cancer	*Breztri/Trixeo* KALOS LOGOS asthma
		Imfinzi + CRT PACIFIC-5 (China) locally-advanced stage III NSCLC	*Imfinzi* + CTx NIAGARA muscle invasive bladder cancer	*Calquence* ECHO 1L MCL	*Lynparza* + Imfinzi DUO-E 1L endometrial cancer	*Fasenra* RESOLUTE COPD
		Imfinzi + VEGF EMERALD-2 adjuvant HCC	*Tagrisso* LAURA stage III EGFRm NSCLC	*Enhertu* DESTINY-Breast11 Neoadjuvant HER2+ breast cancer	*Lynparza* MONO-OLA1 1L BRCAwt ovarian cancer	*Fasenra* FJORD bullous pemphigoid
			Tagrisso + CTx FLAURA2 1L adv EGFRm NSCLC	*Enhertu* DESTINY-Breast05 HER2+ post-neoadjuvant high-risk breast cancer	*Orpathys* + *Imfinzi* SAMETA 1L papillary renal cell carcinoma	*Fasenra* MANDARA EGPA
				Enhertu DESTINY-Breast06 post-ET HER2-low/HR+ breast cancer 2L	*Tagrisso* +/- CTx neoadjuvant NeoADAURA stage II/III resectable EGFRm NSCLC	*Fasenra* NATRON hypereosinophilic syndrome
				Enhertu DESTINY-Breast09 HER2+ breast cancer 1L	*Imfinzi* + domvanalimab (AB154)+ CTx PACIFIC-8 unresectable stage III NSCLC	*Saphnelo* TULIP-SC SLE SC
				Enhertu DESTINY-Gastric04 HER2+ gastric 2L	*Tagrisso* ADAURA2 adjuvant EGFRm NSCLC	*Tezspire* WAYPOINT nasal polyps
				Enhertu DESTINY-Lung04 HER2m NSCLC 1L	*Tagrisso* + *Orpathys* SAFFRON advanced EGFRm NSCLC	*Fasenra* ORCHID nasal polyps
				Imfinzi + CRT KUNLUN locally-advanced ESCC	*Farxiga*/*Forxiga* DAPA-MI prevention of HF and CV death following a myocardial infarction	*Fasenra* HUDSON EG/EGE
				Imfinzi + EV +/- *Imjudo* (tremelimumab) VOLGA MIBC	*Lokelma* DIALIZE-Outcomes	*Ultomiris* (ALXN1210) dermatomyositis
				Imfinzi + FLOT MATTERHORN Neo-adjuvant/adjuvant gastric cancer	*Lokelma* STABILIZE-CKD hyperkalaemia in CKD	*Ultomiris* (ALXN1210) haematopoietic STC-associated TMA
				Imfinzi post-SBRT PACIFIC-4 stage I/II non-small cell lung cancer	roxadustat anaemia MDS	

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

Oncology BioPharmaceuticals Rare Disease

Designations in our pipeline

3 Accelerated approvals

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

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

- *Calquence* CLL ELEVATE-TN, ASCEND (US)
- *Calquence* MCL 1L (US)
- danicopan (ALXN2040) PNH-EVH (US)
- danicopan (ALXN2040) 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)
- *Forxiga* CKD DAPA-CKD (US)
- *Koselugo* NFI type 1 SPRINT (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (CN)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (EU)[1]
- *Tagrisso* adjuvant NSCLC ADAURA (US)
- *Tezspire* asthma NAVIGATOR (US)

9 Fast Track

- CAEL-101 AL amyloidosis (US)
- camizestrant 1L HR+ HER2- ESR1m breast cancer SERENA-6 (US)
- cotadutide NASH (US)
- *Fasenra* EG/EGE HUDSON (US)
- *Forxiga* CKD DAPA-CKD (US)
- *Forxiga* MI RRCT DAPA-MI (US)
- *Lokelma* ESRD DIALIZE-OUTCOMES (US)
- *Beyfortus* RSV mAb-YTE MELODY-MEDLEY (US)
- *Saphnelo* SLE (US)

17 Priority Review

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

29 Orphan

- ALXN1840 WD (US)
- ALXN1840 WD (EU)
- vemircopan (ALXN2050) PNH (US)
- vemircopan (ALXN2050) PNH (EU)
- *Andexxa* Acute Major Bleed (JP)
- CAEL-101 AL amyloidosis (US)
- CAEL-101 AL amyloidosis (EU)
- *Calquence* CLL 1L (US)
- *Calquence* CLL 1L (EU)
- *Calquence* MCL 1L (US)
- danicopan (ALXN2040) PNH (US)
- danicopan (ALXN2040) PNH (EU)
- *Enhertu* HER2+/HER2-low gastric 3L DESTINY-Gastric01 (US)
- eplontersen transthyretin-mediated amyloidosis (US)
- *Fasenra* EG/EGE HUDSON (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)

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.

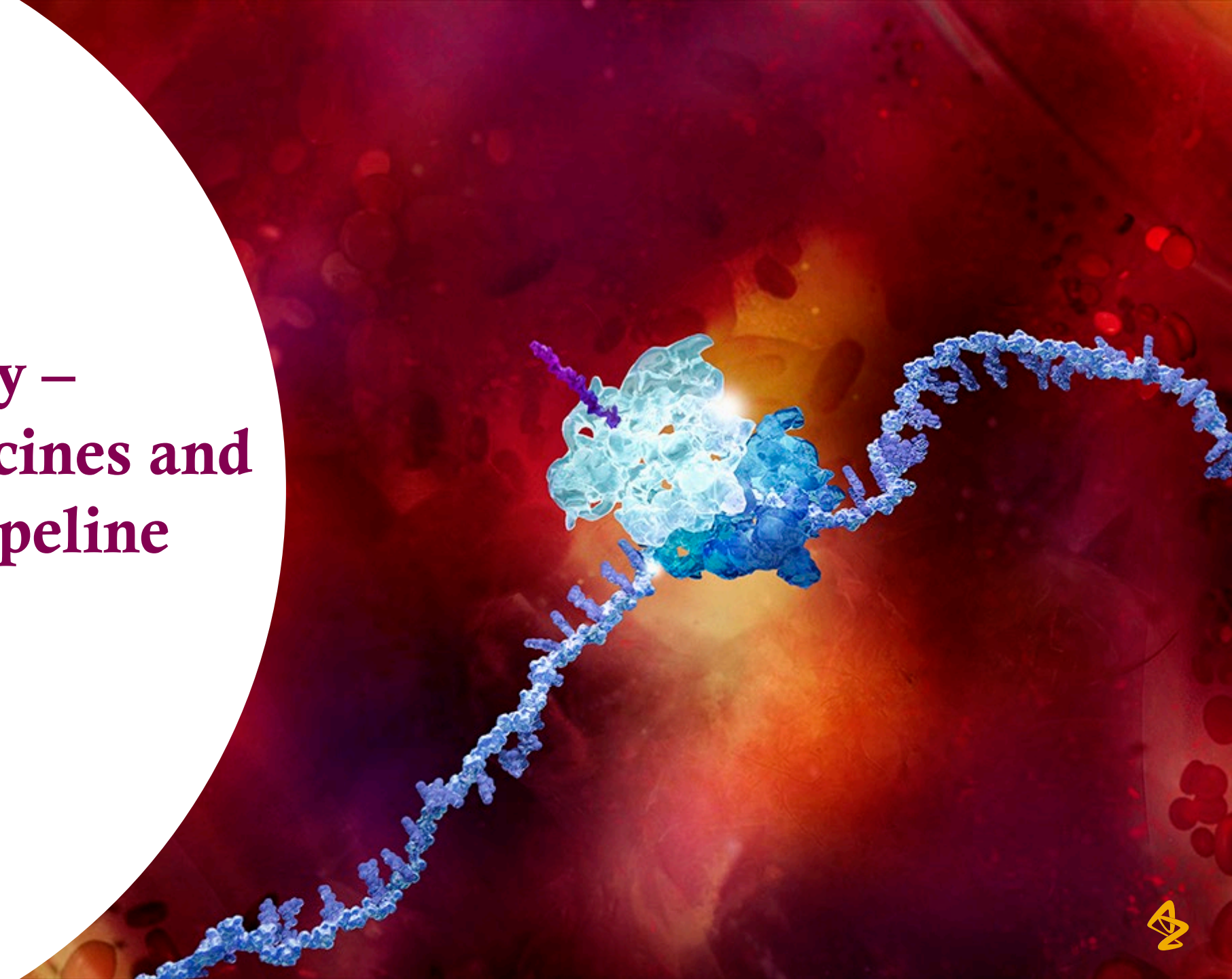
PRIORITY REVIEW DESIGNATION is the US FDA's goal to take action on an application within 6 months.

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.

Note. list excludes designations for projects which have launched in all applicable major markets

Oncology BioPharmaceuticals Rare Disease

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 chemo • Arm 2: placebo • Global trial - 25 countries	• Primary endpoint: DFS • Secondary endpoints: DFS Rate, OS, OS Rate, QoL	• FPCD: Q4 2015 • LPCD: Q1 2019 • Data readout: Q2 2020 • Trial unblinded due to efficacy • DFS primary endpoint 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, DCR	• FPCD: Q4 2018 • Data anticipated: H2 2023
Phase III ADAURA2 NCT05120349	Adjuvant EGFRm NSCLC Stage IA2 - IA3 following complete tumour resection	380	• Arm 1: *Tagrisso* • Arm 2: placebo	• Primary endpoint: DFS • Secondary endpoints: DFS Rate, OS, OS Rate, QoL	• FPCD: Q2 2022 • Data anticipated: >2023

Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III NeoADAURA NCT04351555	Neoadjuvant EGFRm NSCLC	351	• Arm 1: placebo plus pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: *Tagrisso* plus pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 3: *Tagrisso* • Global trial – 23 countries	• Primary endpoint: mPR • Secondary endpoints: cPR, EFS, DFS, OS	• FPCD: Q1 2021 • Data anticipated: >2023
Phase III FLAURA2 NCT04035486	1st-line 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, PK	• 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* plus pemetrexed/carboplatin or pemetrexed/cisplatin • Arm 2: placebo plus pemetrexed/carboplatin or pemetrexed/cisplatin • Global trial	• Primary endpoint: PFS • Secondary endpoints: Intracranial PFS, Extracranial PFS, OS	• FPCD: Q3 2021 • Data anticipated: >2023
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, DoR	• FPCD: Q3 2022 • Data anticipated: >2023
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: >2023

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 endppoint: PFS	• FPCD: Q3 2021 • Data anticipated: >2023
Phase II **SAVANNAH** **NCT03778229**	EGFRm / MET+, locally advanced or metastatic NSCLC who have progressed following treatment with Tagrisso	360	• Protocol v1-6: S 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 include PFS, DoR and OS	• FPCD: Q1 2019 • Data anticipated: >2023 • Initial data readout: Q2 2020
Phase II **ORCHARD** **NCT03944772**	Advanced EGFRm NSCLC patients who have progressed on first line Tagrisso treatment	182	• 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: >2023

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 for Arm 2 vs Arm 3 • Secondary endpoint: PFS for Arm 1 vs Arm 3 , OS	• FPCD: Q1 2019 • LPCD: Q3 2021 • Data anticipated: H2 2022
Phase III EMERALD-2 NCT03847428	Adjuvant therapy in HCC	908	• Arm 1: *Imfinzi* + bevacizumab • Arm 2: *Imfinzi* + placebo • Arm 3: placebo + placebo	• Primary endpoint: RFS for Arm 1 vs Arm 3 • Secondary endpoints: RFS Arm 2 vs Arm 3, OS, RFS at 24m	• FPCD: Q2 2019 • LPCD: Q2 2022 • Data anticipated: H1 2023
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: >2023
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, pCR Arm 1 vs Arm 2	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data anticipated: >2023
Phase III HIMALAYA NCT03298451	HCC 1L	1324	• Arm 1: *Imfinzi* + *Imjudo* • Arm 2: *Imfinzi* • Arm 3: sorafenib	• Primary endpoint: OS • Secondary endpoints: PFS, TTP, ORR	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q4 2021
Phase III TOPAZ-1 NCT03875235	BTC 1L	810	• Arm 1: *Imfinzi* + gemcitabine + cisplatin • Arm 2: placebo + gemcitabine + cisplatin • Global trial	• Primary endpoint: OS • Secondary endpoints: PFS, ORR, DoR	• FPCD: Q2 2019 • LPCD: Q4 2020 • Data readout: Q4 2021
Phase III EMERALD-3 NCT05301842	Locoregional Hepatocellular Carcinoma	525	• Arm A: TACE + T300 + D + Lenva • Arm B: TACE + T300 + D • Arm C: TACE	• Progression Free Survival (PFS) for Arm A vs Arm C • Progression Free Survival (PFS) for Arm B vs Arm C • Overall Survival (OS) for Arm A vs Arm C • Overall Survival (OS) for Arm B vs Arm C	• FPCD: Q2 2022 • Data anticipated: >2023

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 chemo • Arm 2: placebo + platinum-based chemo	• Primary endpoints: pCR, EFS • Secondary endpoint: mPR	• FPCD: Q1 2019 • Data anticipated: H1 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 12m • Arm 2: placebo • Global trial	• Primary endpoint: DFS • Secondary endpoint: OS	• FPCD: Q1 2015 • LPCD: Q1 2020 • Data anticipated: >2023
Phase III PACIFIC-2 NCT03519971	Unresected, locally-advanced NSCLC	300	• Arm 1: *Imfinzi* i.v. Q4W + chemo/RT • Arm 2: placebo + chemo/RT • ex US global trial	• Primary endpoint: PFS • Secondary endpoints: OS, 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: >2023
Phase III PACIFIC-5 NCT03706690	Unresected, locally-advanced NSCLC	360	• Arm 1: *Imfinzi* i.v. Q4W following chemo/RT • Arm 2: placebo following chemo/RT • ex US global trial, China focus	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2019 • Data anticipated: H1 2023

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III PACIFIC-8 NCT05211895 Partnered (Arcus Biosciences)	Unresected, locally-advanced NSCLC	860	• Arm 1: *Imfinzi* + domvanalimab following chemo/RT • Arm 2: *Imfinzi* + PBO following chemo/RT	• Primary endpoint: PFS • Secondary endpoint: OS	• FPCD: Q1 2022 • Data anticipated: >2023
Phase III PEARL NCT03003962	NSCLC 1L	650	• Arm 1: *Imfinzi* Q4W • Arm 2: chemotherapy • Asia trial	• Primary endpoint: OS	• FPCD: Q1 2017 • LPCD: Q1 2019 • Data anticipated: H2 2022
Phase III POSEIDON NCT03164616	NSCLC 1L	1000	• Arm 1: *Imfinzi* + chemo • Arm 2: *Imfinzi* + *Imjudo* + chemo • Arm 3: SoC	• Primary endpoints: OS, PFS	• FPCD: Q2 2017 • LPCD: Q4 2018 • Data readout: Q4 2019 • PFS primary endpoint met • OS data readout Q2 2021
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, OS	• FPCD: Q4 2018 • Data anticipated: H1 2023
Phase III PACIFIC-9 NCT05221840 Partnered (Innate)	Patients with locally advanced (Stage III), unresectable NSCLC, who have not progressed following platinum-based cCRT	999	• Arm 1: *Imfinzi* + oleclumab • Arm 2: *Imfinzi* + monalizumab + placebo • Arm 3: *Imfinzi* + placebo	• Primary endoint: PFS • Secondary endpoint: OS, ORR, DoR, PFS2, TFST	• FPCD: Q2 2022 • Data anticipated: >2023
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 (AZD6738) • 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 outcome: ORR • Secondary outcomes: efficacy including OS, PFS, DCR, and safety and tolerability, DoR	• FPCD: Q1 2018 • Data anticipated: >2023

Imfinzi (PD-L1 mAb)

Lung cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II COAST NCT03822351	Stage III NSCLC unresectable	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
Phase II MAGELLAN NCT03819465	NSCLC 1L	212	• Arm A1: *Imfinzi* • Arm A2: *Imfinzi* + danvatirsen • Arm A3: *Imfinzi* + oleclumab • Arm A4: MEDI5752 • Arm A5: AZD2936 • 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: AZD2936 + chemo	• Primary endpoints: safety & tolerability • Secondary endpoints: ORR, DoR, PFS, OS, PK, ADA	• FPCD: Q1 2019 • Data anticipated: >2023
Phase II NeoCOAST-2 NCT05061550	Early stage, resectable NSCLC (Stage II to Stage IIIA)	140	• Open-label trial • Arm 1: *Imfinzi* + oleclumab + chemotherapy • Arm 2: *Imfinzi* + monalizumab + chemotherapy	• Primary endpoints: pCR, safety	• FPCD: Q2 2022 • Data anticipated: >2023
Phase I/II SCope-D1 NCT04870112	NSCLC SCLC	124	• Open-label, multicentre trial to evaluate the safety, pharmacokinetics, and preliminary efficacy of subcutaneous *Imfinzi*	• Primary endpoints: PK, safety	• FPCD: Q4 2021 • Data anticipated: >2023

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: >2023
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	1063	• Arm 1: *Imfinzi* in combination with gemcitabine + cisplatin, *Imfinzi* maintenance • Arm 2: gemcitabine + cisplatin	• Coprimary endpoints: pCR , 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* versus sunitinib and *Imfinzi* monotherapy	• Primary endpoint: PFS • Secondary endpoints include OS, ORR, DoR and DCR	• FPCD: Q4 2021 • Data anticipated: >2023
Phase III NILE NCT03682068	Bladder cancer 1L	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, pCR • Secondary endpoints: OS	• FPCD: Q4 2021 • Data anticipated: >2023
Phase II BEGONIA NCT03742102	mTNBC 1L	203	• 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, 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

Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DUO-O** **NCT03737643**	Advanced ovarian cancer 1L	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, PFS2	• FPCD: Q1 2019 • Data anticipated: H1 2023
Phase III **DUO-E** **NCT04269200**	Advanced and recurrent endometrial cancer 1L	699	• Arm 1: chemo + *Imfinzi* placebo followed by *Imfinzi* placebo and *Lynparza* placebo • Arm 2: chemo + *Imfinzi* followed by *Imfinzi* + *Lynparza* placebo • Arm 3: chemo + *Imfinzi* followed by *Imfinzi* + *Lynparza* • Global Trial	• Primary endpoint PFS • Secondary endpoints: OS, PFS2, ORR, DoR	• FPCD: Q2 2020 • Data anticipated: H2 2023

Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered (BIG & NRG Oncology)	BRCAm adjuvant breast cancer	1836	• Arm 1: *Lynparza* BID 12-month duration • Arm 2: placebo 12-month duration • Global trial partnership with Breast International Group and National Cancer Institute/NRG Oncology	• Primary endpoint: invasive disease-free survival (iDFS) • Secondary endpoint: 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), PFS (BRCAwt) • Secondary endpoints: OS, TFST, PFS2	• FPCD: Q3 2021 • Data anticipated: >2023

Lynparza (PARP inhibitor)

Other combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **PROpel** **NCT03732820**	Metastatic castration-resistant prostate cancer 1L	904	• Arm 1: *Lynparza* + abiraterone • Arm 2: placebo + abiraterone • Global trial, including China cohort	• 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 resistant/refractory ovarian cancer	680	• Arm 1: chemo • Arm 2: cediranib + *Lynparza* • Arm 3: cediranib • Arm 4: *Lynparza* • US, Canada	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, QoL, 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 endpoints: ORR • Secondary endpoints: DOR, OS, PFS, AE, Prog by CA-125	• FPCD: Q1 2019

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 standard of care 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, CBR • Primary endpoint met	• 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	500	• Randomised open label parallel assignment • *Enhertu* • Ado-trastuzumab emtansine	• Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR, 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 patients	540	• Randomised open label parallel assignment • *Enhertu* • Physician's choice of SoC chemo (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel)	• Primary end point: PFS • Secondary endpoints: OS, DoR, 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 patients 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, BMFI	• FPCD: Q4 2020 • Data anticipated: >2023
Phase III DESTINY-Breast06 NCT04494425 Partnered (Daiichi Sankyo)	HER2-Low, HR+ breast cancer patients whose disease has progressed on endocrine therapy in the metastatic setting	850	• Randomised open label parallel assignment • *Enhertu* • Investigator's choice standard of care chemotherapy (capecitabine, paclitaxel, nab-paclitaxel)	• Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR	• FPCD: Q3 2020 • Data anticipated: H2 2023
Phase III DESTINY-Breast09 NCT04784715 Partnered (Daiichi Sankyo)	HER2-positive, metastatic breast cancer, no prior therapy for advanced or metastatic disease	1134	• Randomised, parallel assignment • *Enhertu* + placebo • *Enhertu* + pertuzumab • Standard of care	• Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR	• FPCD: Q2 2021 • Data anticipated: >2023

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	• Randomized open label parallel assignment • *Enhertu* • *Enhertu*, followed by THP • doxorubicin and cyclophosphamide, followed by THP	• Primary endpoint: pCR • Secondary endpoints: EFS, IDFS, OS	• FPCD: Q4 2021 • Data anticipated: >2023
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, SAE • Secondary endpoints: ORR, PFS, DoR, OS	• FPCD: Q1 2021 • Data anticipated: >2023
Phase Ib **DESTINY-Breast08** **NCT04556773** **Partnered (Daiichi Sankyo)**	HER2-low metastatic breast cancer	185	• Non-randomised open label parallel assignment • *Enhertu* + capecitabine • *Enhertu* + *Imfinzi* + paclitaxel • *Enhertu* + capivasertib • *Enhertu* + anastrozole • *Enhertu* + Faslodex	• Primary endpoints: AE, SAE • Secondary endpoints: ORR, PFS, DoR, OS	• FPCD: Q1 2021 • Data anticipated: H2 2023

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 chemo	• Primary endpoint: OS • Secondary endpoints: ORR, DoR, PFS, DcR, safety	• FPCD: Q2 2021 • Data anticipated: >2023
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 chemo • 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, range of PK endpoints	• 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, 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	• Primary endpoint: ORR • Secondary endpoints: PFS, ORR, DCR, OS, DoR, safety	• FPCD: Q2 2021 • Data anticipated: H2 2023
Phase Ib/II **DESTINY-Gastric03** **NCT04379596** **Partnered (Daiichi Sankyo)**	HER2-overexpressing gastric or gastroeosophogeal junction cancer patients	255	• Open label parallel assignment • Part 1: To determine recommended Phase II combination dose • 5 Arms combine *Enhertu* with standard of care chemotherapies (5-FU, capecitabine, oxaliplatin) and / or durvalumab • Part 2: To assess efficacy of the selected combinations • Arm 2A: Standard chemotherapy (control) • Arm 2B: *Enhertu* monotherapy • Arm 2C: *Enhertu* with chemotherapy • Arm 2D: *Enhertu* with chemotherapy and pembrolizumab • Arm 2E: *Enhertu* and pembrolizumab	• Part 1 Primary endpoint: safety • Part 2 Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS, range of PK endpoints, preence of ADAs	• FPCD: Q2 2020 • Data anticipated: H2 2023

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 Treatment (platinum, pemetrexed and pembrolizumab)	• Primary endpoint: PFS • Secondary endpoints: OS, CNS-PFS, PFS (INV), ORR, DoR, safety; PK, PK/ADA, PRO-Tolerability, PRO-Pulmonary symptoms	• FPCD: Q4 2021 • Data anticipated: >2023
Phase II **DESTINY-Lung01** **NCT03505710** **Partnered (Daiichi Sankyo)**	HER2-over-expressing or mutated, unresectable and/or metastatic NSCLC	170	• Non randomised parallel group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, PFS, OS, 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	150	• Randomised parallel group assignment • Arm 1: *Enhertu* 6.4 mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS, PK	• FPCD: Q1 2021 • Data anticipated: H1 2023
Phase II **DESTINY-PanTumour02** **NCT04482309** **Partnered (Daiichi Sankyo)**	HER2 expressing tumours	280	• Non randomised single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, OS	• FPCD: Q4 2020 • Data anticipated: H2 2023
Phase II **DESTINY-PanTumour01** **NCT04639219** **Partnered (Daiichi Sankyo)**	HER2 mutant tumours	100	• Non-randomised single group assignment • *Enhertu*	• Primary endpoint: ORR • Secondary endpoints: DoR, DCR, PFS, PK	• 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* 6.4 mg/kg • Arm 2: *Enhertu* 5.4mg/kg	• Primary endpoint: ORR • Secondary endpoint: ORR, PFS, OS, DoR, DCR, range of PK endpoints	• FPCD: Q1 2021 • Data anticipated: H1 2023

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 standard of care 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, range of PK endpoints	• FPCD: Q4 2021 • Data anticipated: >2023
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, ORR • Secondary endpoints: DoR, DCR, PFS, TTR, 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, TEAEs • Secondary endpoints: DoR, DCR, PFS, TTR, OS, ORR (investigator)	• FPCD: Q3 2018 • Data readout: Q3 2021

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ACE-CL-007 (ELEVATE-TN)** **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 (independent review committee) assessed ORR, 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 fit	981	• Arm A; *Calquence* + venetoclax • Arm B: *Calquence* + venetoclax + obinutuzumab • Arm C: FCR or BR	• Primary endpoint: IRC PFS (A vs C) • Secondary endpoint: IRC PFS (B vs C); INV PFS (A vs C; B vs C)	• FPCD: Q1 2019 • Data anticipated: >2023
Phase III **ASCEND (ACE-CL-309)** **NCT02970318**	Relapsed/refractory 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, PROs	• FPCD: Q4 2017 • Data readout: Q2 2019 • Primary endpoint met
Phase III **ELEVATE-RR (ACE-CL-006)** **NCT02477696**	Relapsed/refractory high risk CLL	533	• Arm A: *Calquence* • Arm B: ibrutinib	• Primary endpoint: PFS • Secondary endpoints: comparison of incidence of infections, RTs (Richter's Transformation) and atrial fibrillation, OS	• FPCD: Q3 2015 • Data readout: Q1 2021 • Primary endpoint met
Phase III **ECHO (ACE-LY-308)** **NCT02972840**	Previously untreated MCL	626	• 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, OS	• FPCD: Q2 2017 • Data anticipated: H2 2023
Phase III **ESCALADE** **NCT04529772**	DLBCL	600	• *Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine, and prednisone	• Primary endpoints: PFS	• FPCD: Q1 2021 • Data anticipated: >2023
Phase III **D822BC00001** **NCT04075292**	Untreated Chronic Lymphocytic Leukemia	150	• Arm A: *Calquence* • Arm B: Chlorambucil + rituximab	• Primary endpoint: PFS • Secondary endpoint: ORR, DoR	• FPCD: Q1 2015 • Data anticipated: >2023

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib **ACE-LY-106** **NCT02717624**	MCL	70	• *Calquence* in combination with bendamustine and rituximab • Arm A: treatment naive • Arm B: relapsed/refractory • Arm C: treatment naïve: *Calquence* + venetoclax + rituximab	• Primary endpoint: safety	• FPCD: Q2 2016 • LPCD: Q2 2022 • Data anticipated: H2 2022
Phase I/II **ACE-CL-001** **NCT02029443**	CLL/SLL/Richter's transformation	306	• *Calquence* monotherapy • Dose escalation and expansion	• Primary endpoints: safety, PK, PD	• FPCD: Q1 2014 • Data readout: Q4 2021
Phase I **ACE-LY-003** **NCT02180711**	Relapsed/refractory follicular lymphoma	80	• Arm A: *Calquence* • Arm B: *Calquence* + rituximab • Arm C: *Calquence* + rituximab + lenolidomide	• Primary endpoint: safety	• FPCD: Q1 2015 • Data anticipated: >2023
Phase I **ACE-CL-003** **NCT02296918**	CLL/SLL/PLL	69	• *Calquence* + obinutuzumab • Arm A: relapsed/refractory • Arm B: treatment naïve • *Calquence* + venetoclax + rituximab • Arm C: relapsed/refractory • Arm D: treatment naïve	• Primary endpoints: safety, ORR • Secondary endpoints: PD, PFS, TTNT, OS	• FPCD: Q4 2014 • Data readout: Q1 2022

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: >2023
Phase II **NCT04923932** **Partnered (HUTCHMED)**	Locally advanced or metastatic gastric cancer and esophagogastric junction adenocarcinoma patients with MET gene amplifications	75	• Single-arm, multi-cohort, multi-center, open-label trial • *Orpathys*	• Primary endpoint: ORR • Secondary endpoints: PFS, safety	• FPCD: Q3 2021 • Data anticipated: >2023

Capivasertib (AKT inhibitor)

Breast cancer, prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPItello-290 NCT03997123	Locally advanced or metastatic TNBC	924	• Double-blind randomised comparative trial • Arm 1: capivasertib + paclitaxel • Arm 2: placebo + paclitaxel	• Primary endpoint: OS	• FPCD: Q3 2019 • Data anticipated: H2 2023
Phase III CAPItello-291 NCT04305496	2L and beyond in 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: >2023
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: >2023
Phase III CAPItello-280 NCT05348577	mCRPC prostate cancer	790	• Double-blind randomised comparative trial • Arm 1: capivasertib + docetaxel • Arm 2: placebo + docetaxel	• Primary Endpoint: Overall Survival (OS)	• FPCD: Q2 2022 • Data anticipated: >2023

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 (AZD9833) + palbociclib • Arm B: anastrazole + palbociclib	• Primary endpoint: PFS • Secondary endpoint: OS, PFS2	• FPCD: Q1 2021 • Data anticipated: >2023
Phase III SERENA-6 NCT04964934	HR+ HER2- breast cancer	300	• Randomised, double-blind, comparator trial • Arm A: camizestrant (AZD9833) plus palbociclib or abemaciclib • Arm B: anastrazole or letrozole plus palbociclib or abemaciclib	• Primary endpoint: PFS • Secondary endpoint: OS, PFS2	• FPCD: Q3 2021 • Data anticipated: >2023
Phase II SERENA-2 NCT04214288	HR+ breast cancer	240	• Randomised, open-label, parallel-group, multicentre trial • camizestrant (AZD9833) vs. i.m. Faslodex in women with advanced breast cancer.	• Primary outcome: 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	84	• Randomised, open-label, parallel-group, multicentre trial	• Primary outcome: change in ER expression between pre- and on-treatment tumour biopsies	• FPCD: Q4 2020 • Data anticipated: H1 2023
Phase I NCT04541433	HR+ breast cancer	18	• Open-label • anti-tumour activity of camizestrant (AZD9833) in Japanese women with endocrine resistant HR+ HER2- breast cancer that is not amenable to treatment with curative intent.	• Primary outcomes: safety and tolerability • Secondary outcome: PK	• FPCD: Q4 2020 • LPCD: Q1 2022 • Data anticipated: H1 2023
Phase I SERENA-1 NCT03616587	HR+ breast cancer	305	• Escalation phase - open label multicentre trial • camizestrant (AZD9833) • camizestrant (AZD9833) + palbociclib, everolimus, abemeciclib or capivasertib. • Expansion phase - randomised expansion cohort(s) • camizestrant (AZD9833) • camizestrant (AZD9833) + palbociclib, everolimus, abemeciclib or capivasertib.	• Primary outcomes: safety and tolerability • Secondary outcomes: PK, antitumour activity	• FPCD: Q4 2018 • Data anticipated: H1 2023

Camizestrant (AZD9833, next generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04546347**	Healthy volunteers	32	• Randomised, open-label	• Primary outcome: relative bioavailability of different tablet formulations and the effect of food	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q3 2021
Phase I **NCT04818632**	HR+ HER2- breast cancer Chinese patients	30	• Dose escalation • camizestrant (AZD9833) • Dose expansion • camizestrant (AZD9833) • camizestrant (AZD9833) + palbociclib • camizestrant (AZD9833) + everolimus	• Primary outcomes: safety and tolerability, PK • Secondary outcome: antitumour activity	• FPCD: Q1 2021 • Data anticipated: H2 2023

Monalizumab (NKG2a mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III **INTERLINK-1** **NCT04590963** **Partnered (Innate Pharma)**	Recurrent or metastatic HNSCC, 2L	600	• Arm A: monalizumab + cetuximab i.v. • Arm B: placebo + cetuximab i.v. • Global	• Primary: OS • Secondary: PFS, ORR, DoR	• FPCD: Q4 2020 • Data readout: Q3 2022 • Trial stopped for futility

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	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR, TTR, DCR, PK, ADA	• FPCD: Q1 2021 • 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	• Primary endpoints: PFS, OS	• FPCD: Q1 2022 • Data anticipated: >2023
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	• Primary endpoints: PFS, OS	• Initiating
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, ADA	• FPCD: Q1 2021 • LPCD: Q1 2022 • Data anticipated: >2023
Phase I TROPION-Lung02 NCT04526691 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	120	• Open label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy • US, Japan	• Primary endpoints: DLT, safety • Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA	• FPCD: Q4 2020 • Data anticipated: >2023
Phase I TROPION-Lung04 NCT04612751 Partnered (Daiichi Sankyo)	Advanced or metastatic NSCLC	120	• Open label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan + *Imfinzi* +/- platinum chemotherapy • US, Japan	• Primary endpoints: DLT, safety • Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA	• FPCD: Q1 2021 • Data anticipated: >2023

Datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TROPION-Breast01** **NCT05104866** **Partnered (Daiichi Sankyo)**	Inoperable or metastatic HR+, HER2- breast cancer	700	• Open-label, randomised • datopotamab deruxtecan • investigator's choice standard of care chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine)	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR, DCR, PK, ADA	• FPCD: Q4 2021 • Data anticipated: >2023
Phase III **TROPION-Breast02** **NCT05374512** **Partnered (Daiichi Sankyo)**	Locally Recurrent Inoperable or Metastatic Triple-negative Breast Cancer	600	• Open-label, randomised • datopotamab deruxtecan • global trial • investigator's choice of chemotherapy (paclitaxel, nab-paclitaxel, carboplatin, capecitabine, eribulin mesylate)	• Primary endpoints: PFS (BICR), OS • Secondary endpoints: PFS (Inv), ORR, DoR, PK, ADA	• FPCD: Q2 2022 • Data anticipated: >2023
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: Dato-DXd monotherapy • Sub-study 1b: Dato-DXd + *Imfinzi*, • Sub-study 1c: Dato-DXd + AZD5305, • Sub-study 1d: Dato-DXd + durvalumab +AZD5305 • Sub-study 2 (Gastric Cancer); • Sub-study 2a: Dato-DXd + capecitabine, • Sub-study 2b: Dato-DXd + 5-fluorouracil • Sub-study 2c: Dato-DXd + chemotherapy (capecitabine or 5-FU) + nivolumab • Sub-study 3 (mCRPC); • Sub-study 3a: Dato DXd • Sub-study 3b: Dato-DXd + AZD5305 • Sub-study 4 (Ovarian Cancer) • Sub-study 4a: Dato DXd • Sub-study 4b • Arm1: Dato-DXd + carboplatin • Arm2: Dato-DXd + AZD5305 • Sub-study 5 (CRC) • Sub-study 5a: Dato DXd • Sub-study 5b • Arm 1: Dato-DXd + 5-FU + leucovorin (LV) + bevacizumab • Arm 2: Dato-DXd + capecitabine + bevacizumab	• Primary endpoints: ORR, safety	• FPCD: Q3 2022 • Data anticipated: >2023

Datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

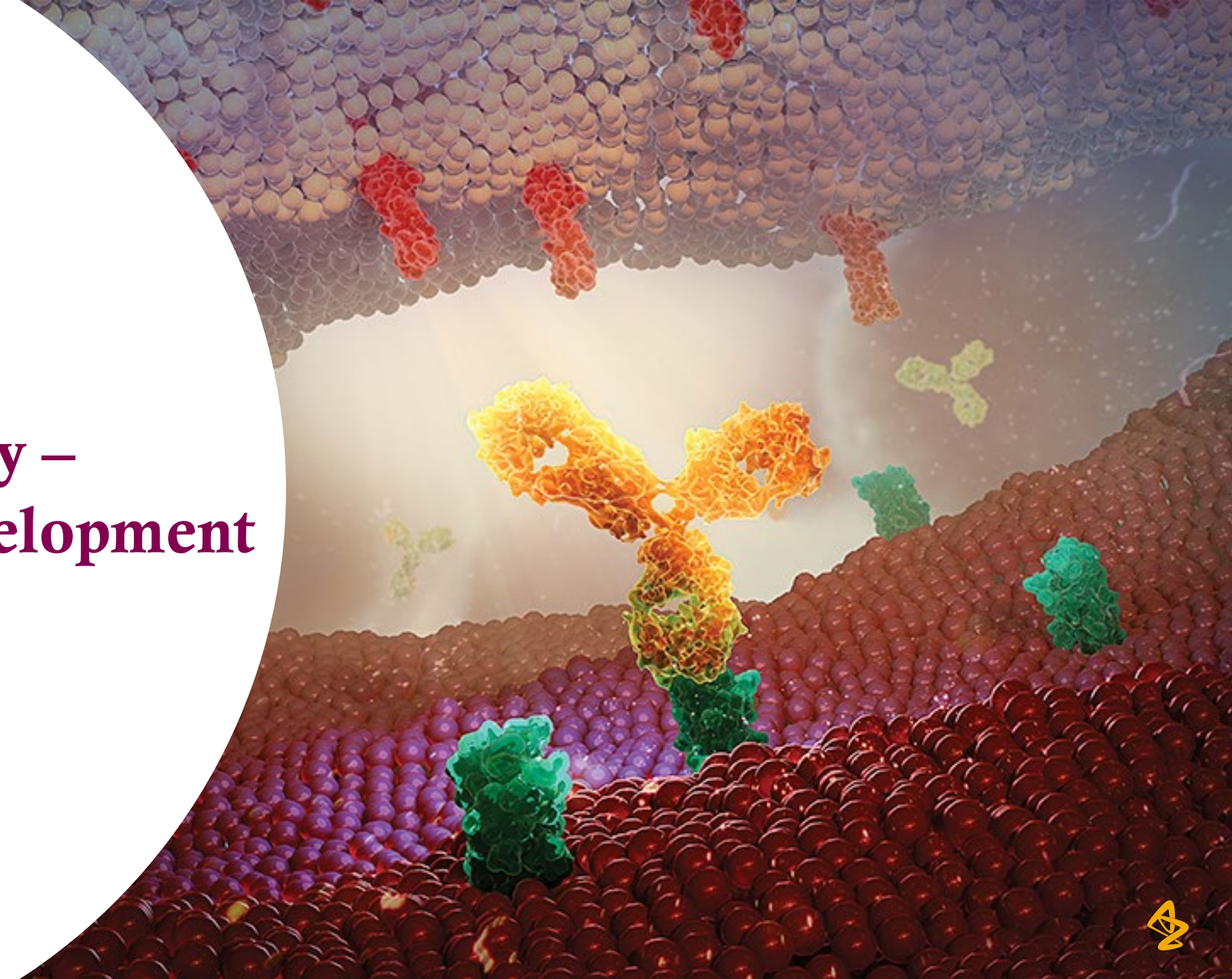
Trial	Population	Patients	Design	Endpoints	Status
Phase I **TROPION-PanTumor01** **NCT03401385** **Partnered (Daiichi Sankyo)**	Subjects with advanced solid tumours NSCLC TNBC HR+ BC HER2-negative gastric/GEJ esophageal Urothelial SCLC	770	• Open label, two-part (dose escalation, dose expansion), sequential assignment • datopotamab deruxtecan • Japan, US	• Primary endpoints: DLT, safety • Secondary endpoints: PK, anti-tumour activity, ADA	• FPCD: Q1 2018 • Data anticipated: >2023 • Early data readout (NSCLC): Q1 2021 • Early data readout (TNBC): Q2 2021

Ceralasertib (AZD6738, ATR inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III LATIFY NCT05450692	Post-IO NSCLC	580	• 2 arm randomised: • Arm 1: Cerala + *Imfinzi* • Arm 2: Docetaxel	• Primary: OS • Secondary: PFS, ORR, DoR, TTR, DCR, PFS2, TTD	• Initiating
Phase II PLANETTE NCT04564027	Solid tumours mCRPC	61	• Cohort A: ceralasertib; ATM-altered AST • Cohort B: ceralasertib; ATM-altered mCRPC • North America and Europe	• Cohort A primary endpoint: ORR • Cohort B primary endpoint: Composite RR	• FPCD: Q1 2021 • Data anticipated: >2023
Phase II MONETTE NCT05061134	Post-IO melanoma 2L+	195	• 2 arm randomised + biopsy sub-study • Arm 1: Cerala + *Imfinzi* • Arm 2: Cerala • Arm 3: Cerala (biopsy sub-study)	• Primary: ORR • Secondary: DoR, TTR, PFS, OS, Safety, biomarkers	• FPCD: Q3 2022 • Data anticipated: >2023
Phase I/II NCT02264678	Solid tumours	330	• Module 1: ceralasertib + carboplatin • Module 2: ceralasertib dose escalation, ceralasertib + *Lynparza* • Module 3: ceralasertib + *Imfinzi* • North America, Europe and South Korea	• Primary endpoints: safety and tolerability. • PK and efficacy	• FPCD: Q4 2014 • Data anticipated: >2023

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 12m • Secondary endpoints: ORR, DoR, PFS	• FPCD: Q1 2022 • Data anticipated: >2023

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	• FPCD: Q2 2021 • Data anticipated: >2023
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 endpoints: Part A: Safety Part B: ORR	• FPCD: Q3 2022 • Data anticipated: >2023

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, MTD • Secondary endpoints: PK and preliminary assessment of anti-tumour activity	• FPCD: Q2 2018 • Data anticipated: >2023

AZD2936 (PD-1/TIGIT Bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ARTEMIDE-01** **NCT04995523** **Partnered (Compugen)**	NSCLC	147	• Open-label, non-randomised dose-escalation and dose-expansion trial: • Part A: Dose escalation in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 Intravenous monotherapy • Part B: Dose expansion in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 intravenous monotherapy • Part C: Dose expansion in CPI Naive NSCLC pts with AZD2936 i.v. monotherapy • Part D: Dose expansion. Design to be confirmed by protocol amendment • Europe, Australia, Taiwan, South Korea, Japan, China and North America	• Part A Dose escalation primary endpoints: safety, RP2D, MTD • Part B dose expansion primary endpoints: Safety and efficacy (ORR) • Part C dose expansion primary endpoints: Safety and efficacy (ORR) • Secondary endpoints: PK, PD (Receptor occupancy), efficacy (DCR, DoR, DRR, PFS)	• FPCD: Q4 2021 • Data anticipated: >2023

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	90	• Open label, non-randomised modular dose confirmation and expansion study in pts with relapsed/ refractory PTCL or cHL • Module 1: Exploring AZD4573 monotherapy treatment in 3 cohorts: • Cohort 1: PTCL, all comers (excluding NKTCL) • Cohort 2: PTCL (NKTCL only) • Cohort 3: cHL • i.v. Route of administration • ~30 centres in 10 countries across North America, Europe, EU and ROW	• Primary endpoints: Efficacy • Secondary endpoint: Safety, PK	• FPCD: Q4 2021 • LPCD: Q3 2023 • Data anticipated: >2023
Phase I/II **NCT04630756**	R/R haematologic malignancies	90	• Open label, non-randomised trial • Module 1 Part A: Dose setting • AZD4573 + *Calquence* (100mg twice daily) combination in DLBCL, all comers; ramp-up across 3 dose levels • Module 1 Part B: Dose expansion • AZD4573 + *Calquence* (100mg twice daily) combination in GCB and non-GCB DLBCL • Module 2 Part A: Dose Confirmation • AZD4573 monotherapy window followed by AZD4573 + acalabrutinib in patients with relapsed or refractory MCL • i.v. route of administration • 10 countries across North America, EU, ROW	• Primary endpoint: Part A: safety • Primary endpoint: Part B: ORR • Secondary endpoints: Safety, PK, anti-tumour activity	• FPCD: Q1 2021 • Data anticipated: >2023
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 relapsed or refractory AML, ALL, high-risk MDS, CMML, CLL and Richter's syndrome • i.v. route of administration • Open in Netherlands, UK, Germany	• Primary endpoints: safety, PK • Secondary endpoint: efficacy	• FPCD: Q4 2017 • LPCD: Q3 2021 • Data anticipated: H2 2022

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)	715	• A modular, open-label, multicentre trial dose escalation • Module 1: AZD5305 • Module 2: AZD5305 + paclitaxel • Module 3: AZD5305 + carboplatin +/- paclitaxel • Module 4: AZD5305 + traztuzumab deruxtecan • Module 5: AZD5305 + datopotamab deruxtecan	• Primary endpoints: safety and tolerability, PK • Secondary endpoints: efficacy	• FPCD: Q4 2020 • Data anticipated: >2023
Phase I/IIa **PETRANHA** **NCT05367440**	Patients with metastatic prostate cancer	126	• 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 in combination with enzalutamide • Arm 2: AZD5305 in combination with abiraterone acetate • Arm 3: AZD5305 in combination with darolutamide.	• Primary endpoints: Safety and Tolerability • Secondary endpoints: PK and Efficacy	• FPCD: Q2 2022 • Data anticipated: >2023

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 pts with AZD7789 intravenous (iv) monotherapy • Part B: Dose expansion in post IO and IO naïve NSCLC pts with AZD7789 iv monotherapy. • North America, Europe	• Primary endpoints: AE, SAE, DLTs, ORR • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA	• FPCD: Q4 2021 • Data anticipated: >2023
Phase I/II **NCT05216835**	Relapsed or Refractory Classical Hodgkin Lymphoma	180	• Cohort A: Dose Escalation - Patients with anti-PD-1/PD-L1 exposed r/r cHL will receive AZD7789 • Cohort B1: Dose Expansion - Patients with anti-PD-1/PD-L1 exposed r/r cHL will receive AZD7789 once the recommended phase 2 dose (RP2D) has been determined • Cohort B2: Dose Expansion - 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 • Cohort B1: AE and ORR • Cohort B2: AE and CRR • Secondary endpoints: • Cohort A: CRR, ORR, DoR, DoCR, PFS, OS, ADA and PK • Cohort B1 and B2: DoR, DoCR, PFS, OS, ADA and PK	• FPCD: Q2 2022 • Data anticipated: >2023

AZD8205 (B7H4 ADC)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05123482	Breast Cancer, Cholangiocarcinoma, Ovarian Cancer, Endometrial Cancer	198	• Open-label, non-randomised dose-escalation and dose-expansion trial	• Primary endpoints: AE, SAE, DLTs, Changes in Lab and PE parameters • Secondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA	• FPCD: Q1 2022 • Data anticipated: >2023

AZD8701 (FOXP3 antisense oligonucleotide)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib NCT04504669 Partnered (Ionis)	Advanced solid tumours	153	• Dose escalation and dose expansion trial • Arm 1: AZD8701 monotherapy • Arm 2: AZD8701 & Durvaluamb combination therapy • i.v. route of administration • US, CA, FR, ES	• Primary endpoints: safety & tolerability • Secondary endpoints: PK, PD, preliminary anti-tumour activity	• FPCD: Q3 2020 • Data anticipated: >2023

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, PD and immunogenicity	• FPCD: Q2 2022 • Data anticipated: >2023

AZD9574 PARP1 Sel BBB inhibitor

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa CERTIS-1 NCT05417594	Patients with Advanced Solid Malignancies	255	• A modular, open-label, dose escalation trial	• Primary endpoints: safety and tolerability of AZD9574 as monotherapy and in combination iwth anticancer agents • Secondary endpoints: PK and efficacy of AZD9574 as monotherapy and in combination iwth anticancer agents	• FPCD: Q3 2022 • Data anticipated: >2023

Capivasertib (AKT inhibitor)

Breast cancer, prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II CAPITAL NCT05008055	Participants with R/R FL, R/R MZL, and R/R MCL	272	• Open-Label, Non-randomized	• Primary Endpoint: ORR, Safety • Secondary Endpoint: DOR, PFS, OS, Safety, PK/PD	• FPCD: Q4 2021 • Data anticipated: H2 2023

IPH5201 (CD39 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04261075** **Partnered (Innate Pharma)**	Advanced solid tumours	204	• 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. • 4 countries - US and 3 in EU.	• Primary endpoints: AE, SAE, DLT • Secondary endpoints: OR, DC, PK, ADA	• FPCD: Q1 2020 • LPCD: Q2 2022 • Data readout: Q4 2022

MEDI9253 (rNDV-IL-12)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04613492**	Advanced solid tumours	86	• Open-label, dose-escalation and expansion trial • MEDI9253 + *Imfinzi*	• Primary endpoint: safety and tolerability • Secondary endpoints: PK, PD, immunogenicity and efficacy	• FPCD: Q4 2020 • Data anticipated: >2023

Oleclumab (CD73 mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II Study 5 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 Australian trial centres	• Primary endpoints: safety and anti-tumour activity • Secondary endpoints include: PFS, PK, immunogenicity, safety and anti-tumour activity	• FPCD: Q2 2018 • Data anticipated: H1 2023

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	• Dose escalation primary endpoints: safety, MTD, RP2D & tolerability. Assess antitumour activity of the combination (ORR) • Secondary endpoints: PK, ADA and antitumour activity (PFS, OR, DoR, DCR, TTR, OS)	• FPCD: Q3 2020 • Data anticipated: >2023
Phase I NCT03530397	Advanced solid tumours	366	• 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	• Dose escalation primary endpoints: safety, tolerability, MTD, OBD and HPDD. • Dose expansion primary endpoint: antitumour activity based on OR • Secondary endpoints: PK, ADA, tumoral baseline PD-L1, antitumour activity (OR, DoR, DCR, PFS, OS)	• FPCD: Q2 2018 • Data anticipated: >2023

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 hemostatic efficacy as rated by an independent adjudication committee • Secondary endpoint: change from baseline in anti-fXa activity	• FPCD: Q2 2019 • Data anticipated: >2023
Phase II **19-515** **NCT04233073**	Urgent surgery	10	• Arm 1: *Andexxa*	• Primary endpoint: proportion of patients with good or excellent intraoperative hemostatic 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 anticipated: H2 2022

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**	Patients with 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* 5 mg + metformin 500 mg XR • Arm 2: *Farxiga*/metformin XR FDC 5/500 mg • Arm 3: *Farxiga* 10 mg + metformin 1000 mg XR • Arm 4: *Farxiga*/metformin XR FDC 10/1000 mg • China only	• Primary endpoint: Plasma AUCinf, AUClast and Cmax of Farxiga and metformin respectively.	• FPCD: Q2 2021 • LPCD: Q2 2021 • Data readout: Q4 2021
Phase I **NCT05266404**	Healthy volunteers	46	• Arm 1: *Farxiga* 10 mg + sitagliptin 100 mg • Arm 2: *Farxiga*/sitagliptin FDC 10/100 mg • Germany only	• Primary endpoint: AUCinf, AUClast and Cmax of Farxiga and sitagliptin repectively.	• FPCD: Q2 2022 • LPCD: Q2 2022 • Data anticipated: H2 2022

Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb **DIALIZE China** **NCT04217590**	Patients with ESRD with hyperkalaemia and on stable haemodialysis	134	• Arm 1: *Lokelma* 5g QD for 8 weeks on non-dialysis days. Option to uptitrate to 10 and 15g QD. • Arm 2: placebo QD for 8 weeks on non-dialysis days • China	• Primary endpoint: proportion of patients who maintain a pre-dialysis serum K between 4.0-5.0 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	• Primary endpoint: maintenance of normokalaemia	• FPCD: Q2 2021 • Data anticipated: H2 2022
Phase III **DIALIZE-Outcomes** **NCT04847232**	Patients with recurrent hyperkalaemia on chronic haemodialysis	2300	• Arm 1: *Lokelma* 5g-15g QD for 4 weeks on non-dialysis days, thereafter adjusted monthly • Arm 2: placebo QD • Global trial – 22 countries	• Primary endpoint: Time to first occurrence of SCD, stroke, or hospitalisation/intervention/ED visit due to arrhythmias	• FPCD: Q3 2021 • Data anticipated: >2023
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 72h, followed by 3 months open-label treatment with *Lokelma* (5g QOD to 15g QD) and uptitration of lisinopril or valsartan. Thereafter, patients are randomized 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 – 13 countries	• Primary endpoint: Total slope (eGFR measurements starting at randomisation) and chronic slope (eGFR measurements starting at • 12 weeks after randomisation)	• FPCD: Q4 2021 • Data anticipated: >2023

Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III Efficacy and Safety of FG-4592 for Treatment of Anemia in Patients With Lower Risk MDS With Low Red Blood Cell Transfusion Burden NCT03263091 Partnered (FibroGen)	Anaemia in lower risk MDS patients	204	• Open label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo • US/global trial	• Primary endpoint: proportion of patients achieving transfusion independence	• FPCD: Q3 2017 • Data anticipated: H1 2023 • Sponsored by FibroGen
Phase II/III Efficacy and Safety of FG-4592 for Treatment of Anemia in Subjects With Lower Risk MDS NCT03303066 Partnered (FibroGen)	Anaemia in lower risk MDS patients	175	• Open label roxadustat lead-in • Arm 1: roxadustat • Arm 2: placebo • China	• Primary endpoint: haemoglobin response	• FPCD: Q2 2018 • Data anticipated: H1 2023 • Sponsored by FibroGen

eplontersen (ligand-conjugated antisense)

ATTR

Trial	Population	Patients	Design	Endpoints	Status
Phase III CARDIO-TTRansform NCT04136171 Partnered with Ionis Pharmaceuticals Inc (Ionis Pharmaceuticals, Inc.)	Patients with hereditary or wild-type Transthyretin-Mediated Amyloid Cardiomyopathy (ATTR CM)	1400	• Arm 1: eplontersen s.c. • Arm 2: placebo	• Primary endpoint: composite Outcome of Cardiovascular (CV) Mortality and recurrent CV clinical events at Week 120 • Secondary endpoints include: 6MWT, KCCQ, CV Events, CV Mortality	• FPCD: Q1 2020 • Data anticipated: >2023
Phase III NEURO-TTRansform NCT04136184 Partnered with Ionis Pharmaceuticals Inc (Ionis Pharmaceuticals, Inc.)	Patients with hereditary transthyretin-mediated amyloid polyneuropathy (hATTR-PN)	168	• Arm 1: eplontersen s.c. • Arm 2: inotersen s.c.	• Primary endpoint: change from baseline in mNIS+7 at Week 66	• FPCD: Q1 2020 • Data readout: Q2 2022 • Met co-primary endpoints

mitiperstat (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb/III ENDEAVOR NCT04986202	HFpEF patients	1485	• Randomised, double blind • Arm 1: 2.5mg mitiperstat • Arm 2: 5mg mitiperstat • Arm 3: placebo • Global trial	• Endpoints: Efficacy and Safety	• FPCD: Q3 2021 • Data anticipated: >2023
Phase IIa SATELLITE NCT03756285	HFpEF patients	41	• Arm 1: mitiperstat • Arm 2: placebo • Global trial – five countries	• Primary endpoint: The change from baseline in MPO activity in %	• FPCD: Q4 2018 • LPCD: Q2 2020 • Data readout: Q4 2020
Phase I NCT04232345	Healthy subjects	32	• MAD trial in Japanese and Chinese patients	• Primary endpoints: safety and tolerability	• FPCD: Q1 2020 • Data readout: Q3 2021
Phase I NCT04407091	Healthy subjects	6	• Open label hADME trial • a single oral dose of [14C] mitiperstat • UK	• Primary endpoints: mass balance, with routes and rates of elimination of [14C]mitiperstat; Metabolite profiling and structural identification; PK and total radioactivity	• FPCD: Q2 2020 • LPCD: Q3 2020 • Data readout: Q3 2021
Phase I NCT04949438	Renal Impaired subjects	20	• Open label • mitiperstat	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2022 • LPCD: Q1 2022 • Data readout: Q2 2022
Phase I NCT05457270	Healthy subjects	30	• Open-label • mitiperstat • 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	• Primary endpoints: relative bioavailability and PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q3 2022 • LPCD: Q3 2022 • Data anticipated: H2 2022

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* • USA, Canada, Japan and additional countries.	• Primary endpoint: Urine albumin creatinine ratio (UACR) • Secondary endpoints: Safety and other efficacy measures	• FPCD: Q4 2019 • Data anticipated: H2 2023

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

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 • 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 • 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 24 • Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations • Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data anticipated: >2023
Phase III LOGOS NCT04609904	Severe asthma	2200	• Randomised, double-blind, double dummy, parallel group and multicentre • 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 • 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 24 • Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations • Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24	• FPCD: Q1 2021 • Data anticipated: >2023
Phase III VATHOS NCT05202262	Moderate asthma	630	• Randomized, double-blind, parallel group and multicentre • 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 • 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 24	• FPCD: Q1 2022 • Data anticipated: >2023

Daliresp/Daxas (PDE4 inhibitor, oral)

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 (PS), age, sex, and year of cohort entry. • The trial is using electronic healthcare databases in the US (Military Health System database), Germany (German Pharmacoepidemiological Research Database), Sweden (national databases including healthcare, death, and demographics data) and Norway.	• Primary endpoint: all-cause mortality (up to five years)	• FPCD: Q1 2017 • LPCD: Q4 2022 • Data anticipated: H2 2022

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 treatment period and open label period • 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: >2023
Phase II **ARROYO** **NCT04612725**	Patients with moderate/severe chronic spontaneous urticaria, and resistant to H1 treatment	155	• Double blind treatment period and open label period • Arm 1: *Fasenra* regimen 1 • Arm 2: *Fasenra* regimen 2 • Arm 3: placebo • 24-week • Global trial	• Primary endpoint: Change from baseline in ISS7 at week 12	• FPCD: Q4 2020 • LPCD: Q2 2022 • Data anticipated: H2 2022

Fasenra (IL-5R mAb)

Gastrointestinal diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MESSINA** **NCT04543409**	Documented diagnosis of EoE Age 12 to 65 years	211	• Double blind treatment period and open label period(s) • Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 24-week • Global trial – 12 countries	• Primary endpoints: histologic response at week 24, change from baseline in DSQ score at week 24	• FPCD: Q4 2020 • LPCD: Q2 2022 • Data readout: Q4 2022 • Trial did not meet one of the two dual-primary endpoints
Phase III **HUDSON** **NCT05251909**	Patients with eosinophilic gastritis and/or gastroenteritis. Age >=12yrs	220	• Double blind treatment period and open label extension • Arm 1: *Fasenra* s.c. • Arm 2: placebo s.c. • 24-week • Global trial	• Dual primary endpoints at week 24: • Proportion of patients achieving a histological response in the stomach and/or in the duodenum • Absolute change in symptoms of EG/EGE	• FPCD: Q1 2022 • Data anticipated: >2023

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 standard of care therapy Age 18-75 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 18-75 years	276	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo Q8W s.c. • 56-week trial • Global trial - 10 countries	• Primary endpoint: Change in endoscopic total nasal polyp score and change in mean nasal blockage score	• FPCD: Q4 2019 • Data anticipated: >2023
Phase III **MANDARA** **NCT04157348**	Patients with relapsing or refractory 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 both weeks 36 and 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- 9-12 countries	• Primary endpoint: Time to first HES worsening/flare	• FPCD: Q3 2020 • Data anticipated: H2 2023

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 D3250C00036 China ICS/LABA Trial (MIRACLE) NCT03186209	Severe, uncontrolled asthma, despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 12-75 years	695	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo s.c. • 56-week trial • Global trial – 4 countries	• Primary endpoint: annual asthma exacerbation rate • Secondary endpoints: assess pulmonary function, asthma symptoms, other asthma control metrics	• FPCD: Q4 2017 • LPCD: Q4 2021 • Data anticipated: H1 2023

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 40-85 years	642	• Double-blind, placebo controlled • 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: >2023
Phase III **MAHALE** **NCT05006573**	Patients With non-cystic fibrosis bronchiectasis (NCFB) with eosinophilic inflammation Age 18 years and older	100	• Double blind treatment period and open label extension trial • Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. • 52-week • Global trial – 17 countries	• Primary endpoint: annualised bronchiectasis exacerbation rate at week 52	• FPCD: Q3 2021 • LPCD: Q3 2022 • Trial discontinued due to strategic portfolio prioritisation

Saphnelo (type I interferon receptor mAb)

Lupus (SLE / LN)

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TULIP LTE** **NCT02794285** **Partnered (BMS)**	Moderate to severe SLE patients	630	• Arm 1: 300mg i.v. *Saphnelo* Q4W for 152 weeks • Arm 2: placebo i.v. Q4W for 152 weeks	• Primary endpoint: extension to evaluate long-term safety and tolerability	• FPCD: Q2 2016 • LPCD: Q4 2018 • Data anticipated: H2 2022
Phase III **TULIP-SC** **NCT04877691** **Partnered (BMS)**	Moderate to severe SLE patients	360	• Arm 1: *Saphnelo* s.c. • Arm 2: placebo s.c. • Global	• Primary endpoint: BICLA at week 52	• FPCD: Q3 2021 • Data anticipated: >2023
Phase III **AZALEA-SLE** **NCT04931563** **Partnered (BMS)**	Moderate to severe SLE patients	328	• Arm 1: 300 mg *Saphnelo* i.v. Q4W • Arm 2: placebo i.v. Q4W • Asia	• Primary endpoint: BICLA at week 52	• FPCD: Q4 2021 • Data anticipated: >2023
Phase III **IRIS** **NCT05138133** **Partnered (BMS)**	Active Proliferative LN patients	360	• Arm 1: *Saphnelo* i.v. • Arm 2: Placebo i.v.	• Complete Renal Response (CRR) at week 52	• FPCD: Q2 2022 • Data anticipated: >2023

Tezspire (TSLP mAb)

Severe uncontrolled asthma, COPD, CRSwNP and EoE

Trial	Population	Patients	Design	Endpoints	Status
Phase III **WAYPOINT** **NCT04851964** **Partnered (AMGEN)**	Severe chronic rhinosinusitis with nasal polyps (CRSwNP) Age 18+	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: >2023
Phase III **DIRECTION** **NCT03927157** **Partnered (AMGEN)**	Severe asthma Age 18-80 years	396	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 52 week 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), asthma control (ACQ-6)	• FPCD: Q3 2019 • Data anticipated: >2023
Phase III **NOZOMI** **NCT04048343** **Partnered (AMGEN)**	Severe asthma 12-80 years	65	• Arm 1: *Tezspire* s.c. • 52 week trial • Local trial - Japan	• Primary endpoint: number of patients with adverse events	• FPCD: Q2 2019 • LPCD: Q1 2020 • Data readout: Q2 2021 • Primary endpoint met
Phase IIa **COURSE** **NCT04039113** **Partnered (AMGEN)**	Moderate to very severe COPD Age 40-80	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: >2023
Phase II **CASCADE** **NCT03688074** **Partnered (AMGEN)**	Severe asthma Age 18-75 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

Tezspire (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR NCT03347279 Partnered (AMGEN)	Severe asthma Age 12-80 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), asthma control (ACQ-6)	• FPCD: Q1 2018 • LPCD: Q3 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III SOURCE NCT03406078 Partnered (AMGEN)	Severe asthma Age 18-80 years	150	• Arm 1: *Tezspire* s.c. • Arm 2: placebo s.c. • 48 week trial • Global trial – 7 countries	• Primary endpoint: Reduction from baseline in daily OCS dose while not losing asthma control • Secondary endpoint: Annual asthma exacerbation rate	• FPCD: Q2 2018 • LPCD: Q4 2019 • Data readout: Q4 2020 • Primary endpoint not met
Phase III DESTINATION NCT03706079 Partnered (AMGEN)	Severe asthma Age 12-80 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 12-80 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 SUNRISE NCT05398263 Partnered (AMGEN)	Severe Asthma Age 18- 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

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	• An 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: >2023
Phase IIb/III **INTREPID** **NCT03759288**	Crohn's disease	928	• A 52-Week, Multicenter, Randomized, Double-blind, Placebo and Active-Controlled, Operationally Seamless Phase 2b/3, 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	• Stage 1 primary endpoint: CDAI remission at week 12 • Stage 1 secondary endpoints: Endoscopic response at week 12, Clinical remission at week 12, CDAI response at week 12, Response and remission at week 52 • Stage 2 primary endpoints: Endoscopic response at week 52, Clinical remission at week 52 •	• FPCD: Q4 2018 • Data anticipated: >2023
Phase II **EXPEDITION** **NCT03616821**	Ulcerative colitis	256	• A 54-Week, Multicenter, Randomized, Double-blind, Placebo controlled, Parallel-group Phase 2 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: >2023

Brazikumab (IL-23 inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04277546**	Ulcerative colitis	165	• A Phase 2 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: >2023
Phase I **NCT05033431**	Healthy volunteers	48	• Open-label Study to Evaluate the Pharmacokinetics, Safety and Tolerability of a Single Dose of Brazikumab Administered by IV Infusion and SC Injection in Healthy Chinese and White Participants	• Primary pharmacokinetic endpoints: • Cmax • AUCinf • AUClast • AUC0-28d	• FPCD: Q4 2021 • Data anticipated: H2 2022

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 80/180 µg prn • BDA MDI 160/180 µg prn • AS (albuterol sulphate) MDI 180 µg 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 80/180 µg QID • BDA MDI 160/180 µg QID • BD MDI 160 µg QID • AS MDI 180 µg QID • 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 160/180 µg • placebo MDI QID • US	• Primary endpoint: The maximum percentage fall from post-dose, pre-exercise baseline in forced expiratory volume in 1 second (FEV1) observed up to 60 minutes post-exercise challenge	• FPCD: Q1 2020 • LPCD: Q3 2020 • Data readout: Q4 2020 • Primary endpoint met

Tozorakimab (IL-33 ligand mAb)

Atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04212169	Adult subjects 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 study: 6 countries	• Primary endpoint: change from baseline at week 16 in Eczema Area and Severity Index (EASI) score • Secondary endpoints: safety and other efficacy measures	• FPCD: Q4 2019 • LPCD: Q2 2022 • Data anticipated: H2 2022
Phase II NCT04570657	Adult participants 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 subjects	36	• Randomized, 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	• Primary endpoint: to characterize the pharmacokinetics of tozorakimab • Secondary endpoints: to evaluate the immunogenicity of tozorakimab	• FPCD: Q3 2021 • LPCD: Q4 2021 • Data readout: Q2 2022

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. + standard of care • Arm 2: Tozorakimab dose 2 s.c. + standard of care • Arm 3: Placebo s.c. + standard of care • 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, change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q1 2022 • Data anticipated: >2023
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. + standard of care • Arm 2: Tozorakimab dose 2 s.c. + standard of care • Arm 3: Placebo s.c.+ standard of care • 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, change in pre-BD FEV1, E-RS:COPD and SGRQ	• FPCD: Q1 2022 • Data anticipated: >2023
Phase II NCT04631016	Adult subjects with COPD and chronic bronchitis	144	• Randomised, double-blind, placebo-controlled, parallel group, proof of concept trial • Arm 1: Tozorakimab s.c. • Arm 2: placebo s.c. • Global study: 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

Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase III PROVENT NCT04625725	Adults having increased risk for inadequate response to active immunisation or having increased risk for SARS-CoV-2 infection	5197	• Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in pre-exposure prophylaxis • Arm 1: *Evusheld* • Arm 2: placebo • *Evusheld*/placebo (2:1) • USA, UK, Belgium, France, 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 conc.	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q3 2021 • Primary endpoint met
Phase III STORM CHASER NCT04625972	Adults with potential exposure to an identified individual with confirmed SARS-COV2 infection and at risk of developing COVID-19	1121	• Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in post-exposure prophylaxis • Arm 1: *Evusheld* • Arm 2: placebo • *Evusheld*/placebo (2:1) • USA and UK	• Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence o: nucleocapsid antibodies, COVID-19 related death, all cause mortality, ADA to Evusheld in serum and AZD7442 serum conc.	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data readout: Q2 2021 • Primary endpoint not met
PHASE III TACKLE NCT04723394	Adults with confirmed mild to moderate SARS-COV2 infection. Symptomatic patients with documented positive SARS-Cov-2 molecular test	910	• Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy for treatment of Covid-19 in non-hospitalised patients • Arm 1: *Evusheld* • Arm 2: placebo • *Evusheld*/placebo (1:1) • UK, Germany, Spain, Italy, Hungary, Russia, US, Mexico, Japan, Poland, Czech Republic, Argentina, Brazil, and Ukraine	• Primary endpoint: efficacy in the prevention of the composite endpoint of either severe COVID-19 or death from any cause through study day 29 • Secondary endpoints: A composite of either death from any cause or hospitalisation for COVID-19 complications or sequelae (Day 1 to Day 169). Determine symptom severity and prevention of respiratory failure	• FPCD: Q1 2021 • LPCD: Q3 2021 • Data readout: Q4 2021 • Primary endpoint met
Phase II ENDURE NCT05375760	Adults and pediatric individuals (≥ 12 years of age weighing at least 40 kg) 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	• Randomized, 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, UK	• Primary: Safety and tolerability; incidence of anitdrug antibodies (ADA) • Secondary: Individual serum concentration; GMTs and GMFR in severe acute respiratory coronavirus-2 neutralizing antibodies	• FPCD: Q2 2022 • LPCD: Q3 2022 • Data anticipated: H2 2023

Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase I PK Co-formulation NCT05166421	Healthy adults Aged ≥ 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) • USA	• Key Endpoints: safety and PK	• FPCD: Q1 2022 • Data anticipated: H2 2023
Phase I TRUST NCT05281601 - (No partner)	Pediatric participants aged ≥ 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 yet to open) • Cohort 3: Severe COVID-19 (cohort yet to open) • *Evusheld* • USA	• Key endpoints: Safety, tolerability and PK	• FPCD: Q1 2022 • Data anticipated: H1 2023

Vaxzevria (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase IV **VICTORIA** **NCT05057897**	Seronegative immunocompromised and immunocompetent individuals who are unvaccinated	360	• Open-label, non-randomized, multi-cohort, multicenter trial. • *Vaxzevria* i.m. • Arm 1: Solid organ transplant • Arm 2: Haematopoietic stem cell transplant • Arm 3: Solid organ cancer patients receiving cytotoxic therapy • Arm 4: Chronic inflammatory disorders • Arm 5: Primary immunodeficiency • Arm 6: Immunocompetent	• Immunogenicity and safety	• FPCD: Q1 2022 • LPCD: Q3 2022 • Data anticipated: H2 2023
Phase III **D8110C00001 (US, global)** **NCT04516746**	Healthy adults Aged 18-65 years	32429	• Adaptive, double-blinded, randomised placebo-controlled trial • *Vaxzevria* • placebo • US, Peru, Chile	• Primary endpoints: efficacy, safety, tolerability, and reactogenicity • Secondary endpoints: immunogenicity	• FPCD: Q3 2020 • LPCD: Q1 2021 • Primary data readout Q1 2021

Nirsevimab (respiratory syncytial virus mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELODY NCT03979313	Healthy infants (born 35 weeks 0 days or greater GA)	3000	• Randomised, Double-blind, placebo-controlled • Arm 1: nirsevimab i.m. • Arm 2: placebo i.m. • Global trial – 31 countries	• Primary endpoints: efficacy • Secondary endpoints: safety, PK, ADA	• FPCD: Q2 2021 (safety cohort) • LPCD: Q4 2021 (safety cohort) • Data Anticipated: H2 2022 (safety cohort) • 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 GA)	800	• Randomised, Double-blind, placebo-controlled • Arm 1: nirsevimab i.m. • Arm 2: placebo i.m. • China Only	• Primary endpoints: efficacy • Secondary endpoints: safety, PK, ADA	• FPCD: Q4 2021 • Data anticipated: >2023
Phase IIb NCT02878330	29-35 WK GA (gestational age) infants	1453	• Randomised, double-blind, placebo-controlled trial • Arm 1: nirsevimab 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 preterm (born 35 weeks 0 day or less GA), CHD and CLD infants eligible to receive Synagis	925	• Randomised, Double-blind, palivizumab-controlled • Arm 1: nirsevimab i.m. • Arm 2: Synagis i.m. • Global trial – 32 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, 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 • nirsevimab i.m. • Route of administration: i.m. • Global trial – 8 countries	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, ADA, efficacy	• FPCD: Q3 2020 • Data anticipated: H1 2023
Phase I China NCT04840849	Healthy Chinese adults, 18-45 years of age	24	• Randomised, Double-blind, placebo-controlled • Arm 1: nirsevimab i.m. • Arm 2: placebo i.m. • Route of administration: i.m. • China only	• Primary endpoint: PK • Secondary endpoints: ADA, safety	• FPCD: Q2 2021 • LPCD: Q2 2021 • Data readout: Q2 2022

BioPharmaceuticals – early-stage development

atuliflapon (FLAP inhibitor)

Cardiovascular disease & Chronic Kidney Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa NCT03317002	CAD	129	• Arm 1: atuliflapon Dose A • Arm 2: atuliflapon Dose B • Arm 3: placebo • Global trial – three countries in Europe	• Primary endpoint: PD effect of atuliflapon by assessment of u-LTE4	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q1 2021

AZD0780 (PCSK9 inhibitor)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05384262	Healthy adults	132	• Randomised, placebo controled SAD/MAD trial	• Primary endpoint: safety and tolerability	• FPCD: Q3 2022 • Data anticipated: H2 2023

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	• 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. • 6 volunteers per cohort receiving AZD2373 and 2 volunteers per cohort receiving placebo. • Arm 1: AZD2373 s.c. • Arm 2: Placebo s.c. • US	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters. Effect of SC MAD administrations of AZD2373 on plasma concentrations of APOL1 protein. Determine APOL1 G0, G1, G2 allele genotype status in study participants.	• FPCD: Q2 2022

AZD2693 (antisense oligonucleotide)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04142424	Healthy volunteers	72	• SAD. 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	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2019 • LPCD: Q3 2021 • Data readout: Q1 2022
Phase I NCT04483947	NASH/NAFLD F0-F3	67	• MAD. 3 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • US	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q2 2021 • Data anticipated: H2 2023
Phase I NCT05107336	Healthy Participants	44	• MAD. 4 cohorts receiving AZD2693 and placebo in each cohort • Arm 1: AZD2693 s.c. • Arm 2: placebo s.c. • JP	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2021 • Data anticipated: H2 2023

AZD3366

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04588727**	Healthy volunteers	103	• SAD trial • Part A • 7 cohorts with 6 volunteers receiving AZD3366 and 2 volunteers receiving placebo in each cohort; three cohorts with Japanese subjects 5 receiving AZD3366 • 1 receiving placebo; 1 Chinese cohort of 6 receiving AZD3366 2 receiving placebo • Arm 1: AZD3366 • Arm 2: placebo • Part B • 12 subjects • Arm 1: AZD3366 + *Brilinta* + ASA • Arm 2: placebo + *Brilinta* + ASA	• Primary endpoints: safety and tolerability • Secondary endpoint: PK parameters	• FPCD: Q4 2020 • LPCD: Q1 2022 • Data readout: Q3 2022

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04630067**	SAD – Healthy volunteers MAD – Heart failure	104	• Multicentre single and multiple ascending dose study • Part A SAD 6 cohorts • Arm 1: AZD3427 • Arm 2: placebo • Part B MAD • Arm 1: AZD3427 • Arm 2: placebo • US	• Primary endpoints: safety and tolerability	• FPCD: Q4 2020 • LPCD: Q3 2022 • Data anticipated: H2 2022

AZD5462 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04994106**	SAD – Healthy volunteers MAD – Healthy volunteers	0	• Single centre single and multiple ascending dose study • Part A SAD 8 cohorts • Arm 1: AZD5462 • Arm 2: placebo • Part B MAD 5 cohorts • Arm 1: AZD5462 • Arm 2: placebo • US	• Primary endpoints: safety and tolerability	• FPCD: Q4 2021 • LPCD: Q3 2022 • Data anticipated: H1 2023

AZD6234 (long-acting amylin)

Obesity with related comorbidities

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

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	• Primary endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q4 2021 • Data anticipated: H1 2023

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* 10 mg • Arm 2: AZD9977 B + *Farxiga* 10 mg • Arm 3: AZD9977 C + *Farxiga* 10 mg • Arm 4: *Farxiga* 10 mg • 12 weeks • Trial conducted in 19 countries globally	• 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: H1 2023

Cotadutide (GLP-1-glucagon agonist)

Diabetes/CKD, NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II/III **PROXYMO ADVANCE** **NCT05364931**	Patients with F2/F3 biopsy confirmed NASH	1860	• Phase IIb/III Randomized, Double-blind, Placebo-controlled	• 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 • Data anticipated: >2023
Phase II **NCT04515849**	Chronic kidney disease with type 2 diabetes mellitus	225	• Arm 1: cotadutide 100 micrograms • Arm 2: cotadutide 300 micrograms • Arm 3: cotadutide 600 micrograms • Arm 4: semaglutide • Arm 5: placebo	• Primary endpoint: efficacy change in UACR • Secondary endpoints: Change in HbA1c; change in glucose measured by CGM; effects on body weight; safety, tolerability, Immunogenicity	• FPCD: Q4 2020 • LPCD: Q3 2021 • Data readout: Q2 2022

MEDI6570

Cardiovascular

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT04610892**	Post MI	400	• Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure (HF) 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, Russia	• Primary endpoints: efficacy and safety	• FPCD: Q4 2020 • LPCD: Q4 2022 • Data anticipated: >2023

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	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, ADA	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q2 2022

mitiperstat (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05236543**	Healthy Subjects	14	• Open label • mitiperstat • mitiperstat and Itraconazole • UK	• Primary endpoints: PK parameters • Secondary endpoints: safety and tolerability	• FPCD: Q1 2022 • LPCD: Q3 2022 • Data anticipated: H2 2022

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* 10 mg once daily • Arm 2: zibotentan dose B + *Farxiga* 10 mg once daily • Arm 3: *Farxiga* 10 mg + placebo once daily • Global study	• Primary endpoint: • Change in log-transformed UACR from baseline to week 12 zibotentan dose B/dapagliflozin 10 mg versus dapagliflozin 10 mg. • • Secondary endpoints: • Change in Log-transformed UACR from baseline to Week 12 zibotentan dose A/dapagliflozin 10 mg versus dapagliflozin 10 mg. • 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: H1 2023
Phase I **NCT04991571**	Healthy volunteers	28	• Part 1 of the study is intended to collect samples for Metabolites in Safety Testing (MIST) analysis after administration of multiple doses of zibotentan. • Part 2 of the study is designed to evaluate the relative bioavailability of zibotentan and dapagliflozin after dosing with two different fixed-dose combination (FDC) formulations and dosing with separate formulations of zibotentan and dapagliflozin • US based study	• Part 1: • Metabolites in Safety Testing MIST analysis. No PK statistical analysis will be performed. • Part 2: • PK parameters	• FPCD: Q3 2021 • LPCD: Q4 2021 • Data readout: Q2 2022

Zibotentan (endothelin receptor antagonist)

Liver Cirrhosis with Features of portal hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05112419**	Cohort 1: Moderate hepatic impairment/moderate renal impairment patients Cohort 2: Healthy volunteers	24	• Single dose, single centre, open label, parallel group study to investigate the pharmacokinetics, safety and tolerability of 5 mg zibotentan • Single country study (Bulgaria)	• Primary Endpoints: • Area under plasma concentration-time curve from time zero to infinity (AUCinf) • Area under the plasma concentration-curve from time zero to time of last quantifiable concentration (AUClast) • Maximum observed plasma concentration (Cmax)	• FPCD: Q4 2021 • LPCD: Q4 2021 • Data readout: Q2 2022

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 pharmacokinetics (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 • US, Hungary, Japan, Netherlands, Poland, Australia, Bulgaria, Croatia, France, Germany, Italy, South Korea, Spain, UK, Romania, Sebia, Slovakia, Slovenia, South Africa	• Primary endpoint; Time to first CompEx Asthma event (Composite endpoint for Exacerbations)	• FPCD: Q2 2022 • Data anticipated: >2023

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	• Part 1 primary endpoints: safety and tolerability, PK • Part 2 primary endpoint: change in FEV1	• FPCD: Q2 2021 • Data anticipated: H2 2023

AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04769869**	Healthy subjects 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	• Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, FENO	• FPCD: Q4 2021 • Data anticipated: H1 2023

AZD5055 (oral porcupine inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05134727**	Healthy subjects	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 • Data anticipated: H1 2023

AZD7798 (humanized monoclonal antibody)

Crohn's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05452304**	Healthy subjects	64	• Single Ascending Dose • Arm1: AZD7798 • Arm2: Placebo	• Primary endpoints: Safety and tolerability • Secondary endpoints: pharmacokinetics (PK) and immunogenicity	• FPCD: Q3 2022 • Data anticipated: H2 2023

AZD8630 (inhaled TSLP)

Asthma

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

AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04076540 Partnered with Eolas Therapeutics Inc and NIH (Partnered with Eolas Therapeutics Inc and NIH)	Healthy volunteers	48	• Randomised, double blind, SAD trial • Arm 1: AZD4041 • Arm 2: placebo • US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q4 2019 • Data readout: Q4 2021 • Primary endpoint met
Phase I NCT05209334 Partnered with Eolas Therapeutics Inc and NIH (Partnered with Eolas Therapeutics Inc and NIH)	Healthy volunteers	36	• Randomised, double blind MAD trial • Arm 1: AZD4041 • Arm 2: placebo • Canada only	• Primary Endpoints: safety and tolerability • Secondary endpoint: PK	• FPCD: Q1 2022 • LPCD: Q2 2022 • Data anticipated: H2 2022

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	• FPCD: Q4 2019 • Data readout: Q2 2022

MEDI1341 (alpha-synuclein mAb)

Parkinson's disease

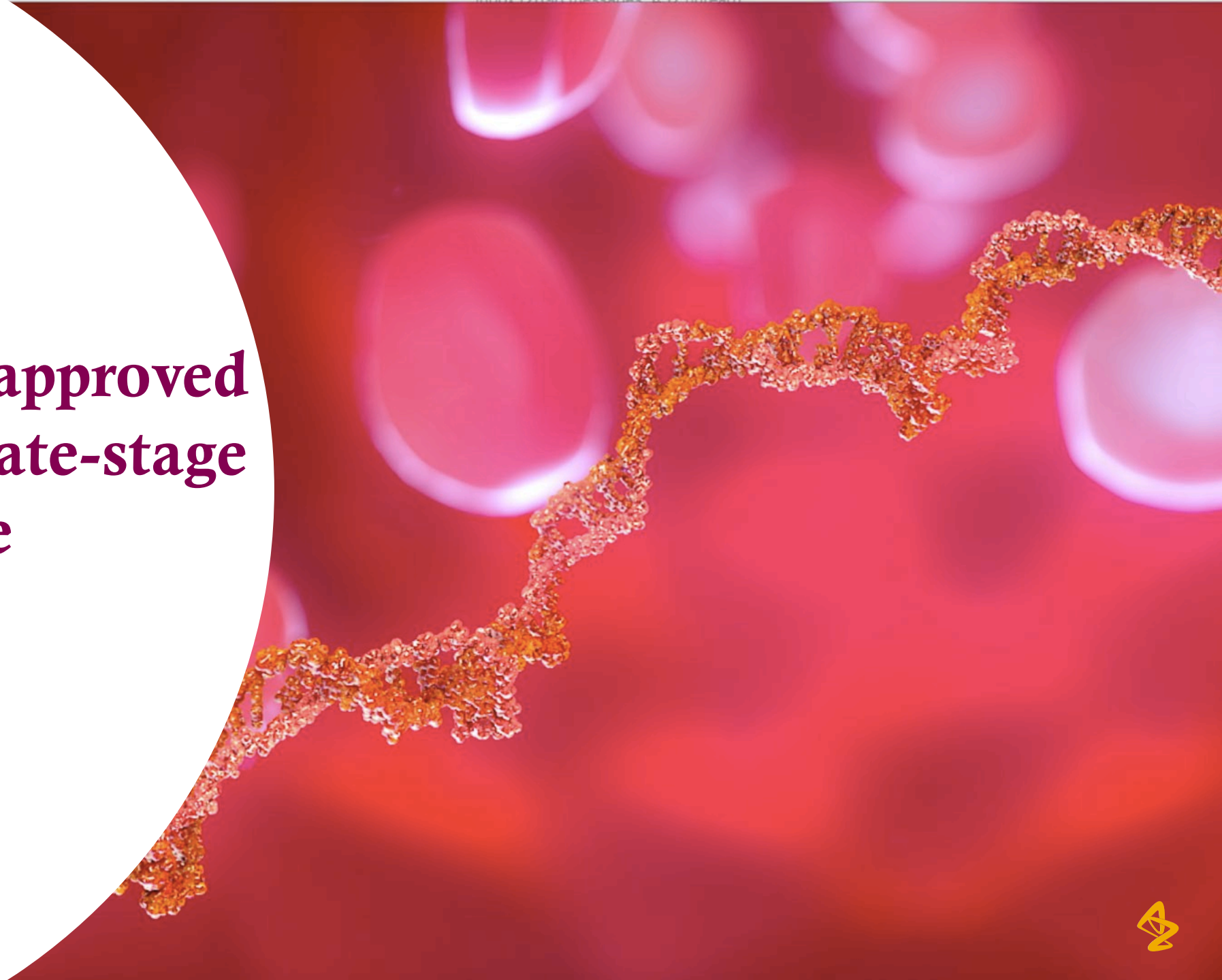
Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03272165 Partnered (Takeda)	Healthy volunteers	48	• SAD trial • Arm 1: MEDI1341 i.v. • Arm 2: placebo i.v. • US only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q4 2017 • LPCD: Q4 2020 • Data readout: Q4 2021
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, PD	• FPCD: Q3 2020 • LPCD: Q3 2021 • Data readout: Q4 2022

MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT04675034**	Painful osteoarthritis of the knee	300	• MAD trial • Arm 1: MEDI7352 s.c • Arm 2: placebo s.c • Global (7 countries)	• Primary endpoint: dose response • Secondary endpoints: safety, tolerability, PK, PD, ADA	• FPCD: Q1 2021 • 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, PD	• FPCD: Q4 2018 • Data anticipated: >2023
Phase I **NCT02508155**	Painful osteoarthritis of the knee	160	• SAD & MAD trial • Arm 1: MEDI7352 i.v. • Arm 2: placebo i.v. • Arm 3: MEDI7352 s.c • Arm 4: placebo s.c • Europe only	• Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD	• FPCD: Q1 2016 • LPCD: Q4 2020 • Data readout: Q2 2021
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, PD, ADA	• FPCD: Q2 2021 • LPCD: Q3 2021 • Data readout: Q4 2021

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, randomized, 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. • Key secondary endpoint: Chronic PN-pain intensity (change from baseline) on Koselugo vs placebo	• FPCD: Q4 2021 • Data anticipated: >2023

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 endpoint: 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 (1-6 years old) diagnosed with NF1 with symptomatic, inoperable PN Must have at least one measurable PN, defined as a PN of at least 3 cm measured in one dimension	38	• Single-Arm - Selumetinib • Open Label	• 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) (each cycle is 28 days)] • - Adverse Events graded by CTCAE Ver 5.0 [Time Frame: from screening until 30 days after last dose]	• FPCD: Q1 2022 • Data anticipated: H2 2023
Phase I Japan PK / Safety trial NCT04495127	Paediatric inoperable NF1-PN patients	12	• Open-label trial • *Koselugo* in Japanese paediatric NF1-PN patients	• Primary endpoint: safety • Secondary endpoints: PK, anti-tumour effect	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data readout: Q4 2021
Phase I China PK / Safety / Efficacy trial NCT04590235	Pediatric (2-17 years old), adult NF1	32	• Single arm trial with 3 phases; • Dose confirmation phase (n=6 for 3 cycles), • Expansion phase (24mths post LSD) • Long term follow up (60mths post LSD)	• Primary endpoints: safety, tolerability and PK • Secondary endpoints: efficacy (ORR, DoR; TTR; PFS)	• FPCD: Q1 2021 • Data anticipated: H2 2023
Phase I Food Effect/Gi Tolerability Study 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 versus the same dose given in a fasted state.	• Primary endpoints: PK (steady state systemic exposure), safety (especially GI toxicity)	• FPCD: Q3 2021 • Data anticipated: H1 2023

Soliris (anti-complement C5 mAb)

Neurology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ECU-GBS-301** **NCT04752566**	Guillain Barré syndrome	57	• Arm 1: *Soliris* i.v. once weekly for 4 weeks • Arm 2: Placebo • Japan-only	• Primary endpoint: Time to first reaching a Hughes functional grading scale score ≤ 1	• FPCD: Q1 2021 • Data readout: Q3 2022 • Primary enpoint not met

Ultomiris (anti-complement 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 with 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: >2023
Phase III **ALXN1210-TM-314** **NCT04557735**	Paedeatric thrombotic microangiopathy associated with haematopoietic stem cell transplant	40	• Arm 1: *Ultomiris* administered once every 4-8 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: >2023
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: Both cohorts, percentage change in proteinuria from baseline to Week 26 • Secondary endpoints: Both cohorts, percentage change in proteinuria from baseline to Week 50	• FPCD: Q1 2021 • Data anticipated: >2023

Ultomiris (anti-complement 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	48	• Arm 1: *Ultomiris* • Arm 2: placebo	• Primary endpoint : improvement response on International Myositis Assessment And Clinical Studies-Total Improvement Score (IMACS-TIS)	• FPCD: Q4 2021 • Data anticipated: >2023
Phase II/III **ALXN1210-NMO-317** **NCT05346354**	Neuromyelitis Optica Spectrum Disorder	12	• Arm 1: *Ultomiris* Q8W	• Primary Endpoint: Change From Baseline in the Annualized Relapse Rate at Week 50	• FPCD: Q3 2022 • Data anticipated: >2023

acoramidis (ALXN2060)

ATTR-CM

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2060-TAC-302** **NCT04622046**	ATTR-CM	22	• Arm 1: 800 mg acoramidis (ALXN2060) administered twice daily	• Primary endpoint: Change from Baseline to Month 12 of Treatment In Distance Walked During the Six-minute Walk Test, All-cause mortality and cardiovascular-related hospitalization over a 30-month period	• FPCD: Q4 2020 • Data anticipated: >2023

ALXN1840 (bis-choline tetrathiomolybdate)

Wilson disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **WTX101-301 FoCus** **NCT03403205**	Wilson Disease	215	• Arm 1: ALXN1840 was administered orally for 48 weeks at doses ranging from 15 milligrams (mg) every other day (QOD) up to a titrated dose of 60 mg daily. • Arm 2: Standard of care	• 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	10	• Arm 1: Participants will be administered ALXN1840 at a dose of 15 milligrams (mg)/day on Day 1 through Day 28 and then increased to 30 mg/day on Day 29 through Day 39	• Primary endpoint: mean daily copper balance	• FPCD: Q3 2020 • Data anticipated: H2 2022

ALXN1840 (bis-choline tetrathiomolybdate)

Wilson disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II **ALXN1840-WD-205** **NCT04422431**	Wilson Disease	31	• Arm 1: ALXN1840, Participants will be initiated at 15 milligrams once daily, then the dose will be increased to 30 milligrams once daily at Week 6.	• Primary endpoint: change from baseline at Week 48 in liver copper concentration	• FPCD: Q4 2020 • Data anticipated: H2 2022

CAEL-101 (fibril-reactive mAb)

AL amyloidosis

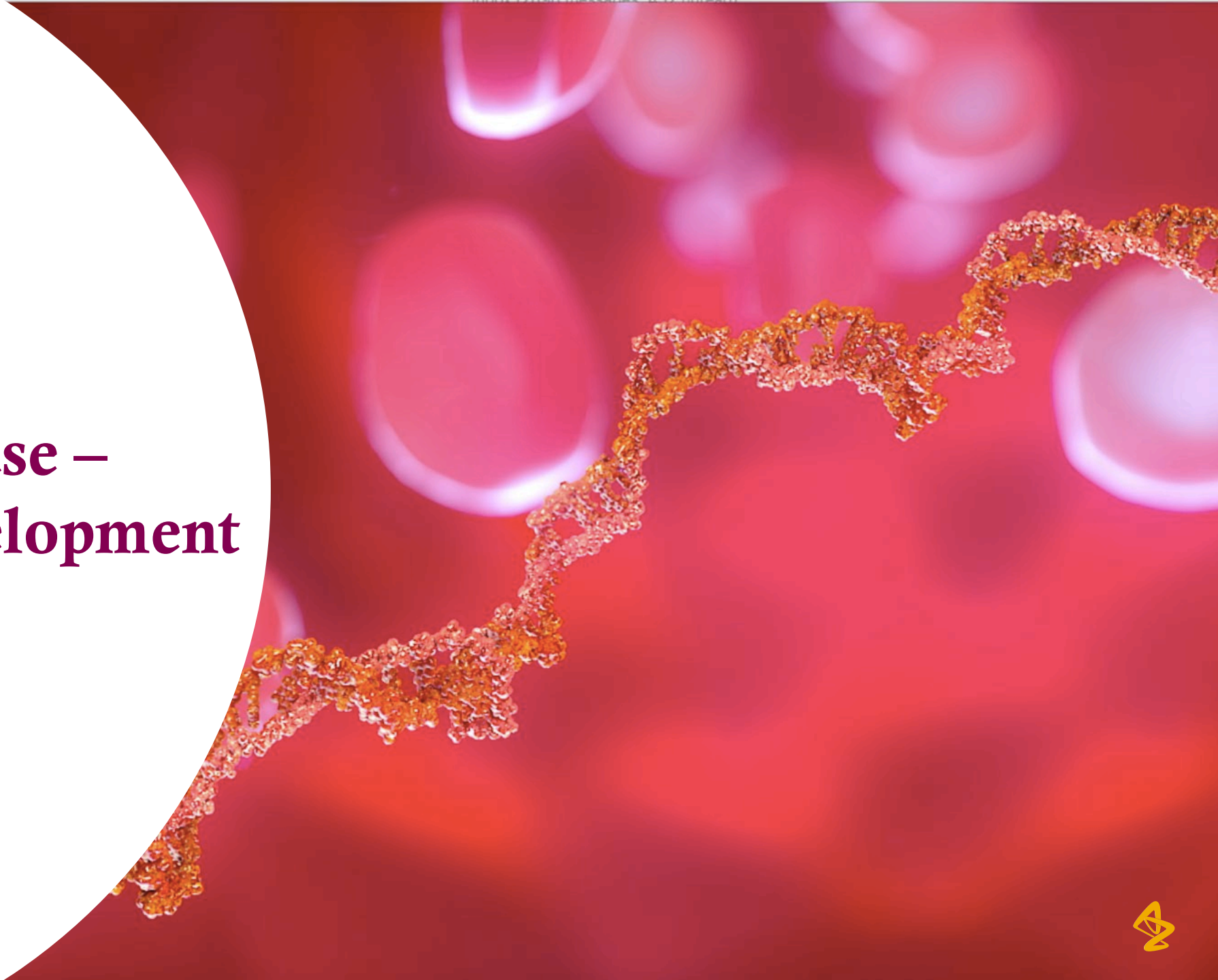
Trial	Population	Patients	Design	Endpoints	Status
Phase III CAEL101-302 NCT04512235	Mayo stage IIIa amyloidosis	267	• Arm 1: CAEL-101 combined with SoC for plasma cell dyscrasia (PCD) • Arm 2: placebo combined with SoC for PCD	• Primary: time from first dose of trial drug until death or end of trial • Secondary: change in distance walked during a six-minute walk test, and quality of life measures	• FPCD: Q4 2020 • Data anticipated: >2023
Phase III CAEL101-301 NCT04504825	Mayo stage IIIb amyloidosis	124	• Arm 1: CAEL-101 combined with SoC for PCD • Arm 2: placebo combined with SoC for PCD	• Primary: time from first dose of trial drug until death or end of trial • Secondary: change in distance walked during a six-minute walk test, and quality of life measures	• FPCD: Q1 2021 • Data anticipated: >2023
Phase II CAEL101-203 NCT04304144	Mayo Stage I, Stage II and Stage IIIa amyloidosis	25	• Arm 1: CAEL-101 combined with SoC CyBorD • Arm 2: placebo combined with SoC CyBorD and daratumumab	• Primary: dose toxicity – occurrence of dose limiting toxicity (DLT) during the first 4 weeks of therapy • Secondary: area under the plasma curve concentration versus time curve (AUC)	• FPCD: Q1 2020 • Data anticipated: >2023

danicopan (ALXN2040 factor D inhibitor)

Haematology & ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ALXN2040-PNH-301** **NCT04469465**	PNH with clinically relevant extravascular haemolysis	84	• Arm 1: danicopan (ALXN2040) + 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 II **ALXN2040-GA-201** **NCT05019521**	Geographic atrophy	330	• Arms 1-3: danicopan (ALXN2040) dosed at 100-400mg daily • 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: >2023

Rare Disease – early-stage development



ALXN1820 (anti-properdin)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1820-HV-101** **NCT04631562**	Healthy Volunteers	59	• Arm 1: ALXN1820 administered s.c. or i.v., multiple ascending doses • Arm 2: Placebo	• Primary: Participants with TEAEs	• FPCD: Q1 2021 • Data anticipated: H1 2023

ALXN1850 (next generation asfotase alfa)

Hypophosphatasia

Trial	Population	Patients	Design	Endpoints	Status
Phase I **ALXN1850-HPP-101** **NCT04980248**	HPP	15	• Arm 1: ALXN1850, 3 cohorts at low, medium, and high dosages	• Primary: Incidence of TEAEs and TESAEs	• FPCD: Q3 2021 • Data anticipated: H2 2022

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

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: >2023

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 (ALXN2040)	• Primary endpoint: Change in haemoglobin relative to baseline • Secondary endpoints: Number of participants who have transfusion avoidance, Change in lactate dehydrogenase relative to baseline	• FPCD: Q4 2019 • Data readout: Q3 2023
Phase II **ALXN2050-NEPH-201** **NCT05097989**	Lupus Nephritis (LN) or Immunoglobulin A Nephropathy (IgAN)	126	• Arm 1: LN Cohort: vemircopan 180 mg • Arm 2: LN Cohort: vemircopan 120 mg • Arm 3: LN Cohort: Placebo • Arm 4: IgAN Cohort: vemircopan 180 mg • Arm 5: IgAN Cohort: vemircopan 120 mg • Arm 6: IgAN Cohort: Placebo	• Primary Endpoint: Both Cohorts: Percentage Change In Proteinuria From Baseline To Week 26	• FPCD: Q3 2022 • Data anticipated: >2023
Phase II **ALXN2050-gMG-201** **NCT05218096**	Generalized myasthenia gravis (gMG)	70	• Arm 1: ALXN2050 180mg • Arm 2: ALXN2050 120mg • Arm 3: placebo followed by ALXN2050	• Primary endpoint: Myasthenia Gravis Activities of Daily Living (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: >2023
Phase I **ALXN2050-HV-109** **NCT05259085**	Impaired Hepatic Function	36	• Arm 1: Mild IHF, 120 mg vemircopan orally twice daily on Days 1 through 3, 120 mg orally on the morning of Day 4 • Arm 2: Moderate IHF, 120 mg vemircopan orally twice daily on Days 1 through 3, 120 mg orally on the morning of Day 4 • Arm 3: Severe IHF, 120 mg vemircopan orally twice daily on Days 1 through 3, 120 mg orally on the morning of Day 4 • Arm 4: Healthy Control, 120 mg vemircopan orally twice daily on Days 1 through 3, 120 mg orally on the morning of Day 4 •	• Primary Endpoint 1: Area Under The Concentration-time Curve From Time 0 To The 12-hour Time Point (AUC0-12) Of Plasma vemircopan After Steady-state • Primary Endpoint 2: Area Under The Concentration-time Curve Calculated To The Last Observable Concentration At Time t (AUCt) Of Plasma vemircopan After Steady-state • Primary Endpoint 3: Maximum (Peak) Steady-state Plasma Concentration Of vemircopan (Cmax,ss) • Primary Endpoint 4: Time To Reach Maximum (Peak) Plasma Concentration Following vemircopan Administration At Steady-state (Tmax,ss)	• FPCD: Q2 2022 • Data anticipated: H2 2023

ALXN2080 oral factor D

Complement-mediated disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT05428696**	Healthy participants	100	• SAD/MAD trial	• Primary endpoints: Safety and Tolerability, PK, PD	• FPCD: Q3 2022

gefurulimab (ALXN1720 anti-C5 bi-specific minibody)

Neurology and nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1720-HV-101 NCT04920370	Healthy Volunteers	96	• Single & Multiple Ascending Dosing of gefurulimab via s.c. and i.v. administration • Placebo	• Primary: Incidence of TEAEs and SAEs	• FPCD: Q3 2019 • Data readout: Q2 2022
Phase I ALXN1720-NEPH-102 NCT05314231	Proteinuria	12	• Arm 1: gefurulimab, SC infusion at a dose of 1500 mg	• Primary Endpoint: Serum Concentration of gefurulimab [Time Frame: Day 1 (0.5 hours predose and postdose) and postdose on Days 2, 3, 8, 15, 29, 43, and 57]	• FPCD: Q2 2022 • Data anticipated: H2 2023

List of abbreviations

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

List of abbreviations

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

List of abbreviations

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