AstraZeneca

Clinical trials appendix

Q1 2021 results update

Movement since Q4 2020 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
		NME **camizestrant (AZD9833) + palbociclib SERENA-4** selective oestrogen receptor degrader + CDK4/6 inhibitors 1st-line HR+ HER2- breast cancer **datopotamab deruxtecan# TROPION-Lung01** TROP2 targeting antibody drug conjugate 2L+ NSCLC without actionable genomic mutations **Lifecycle Management** ***Enhertu*# (platform) DESTINY-Breast07** HER2 targeting antibody drug conjugate HER2+ breast cancer ***Enhertu*# (platform) DESTINY-Breast08** HER2 targeting antibody drug conjugate HER2low breast cancer ***Fasenra* FJORD** IL-5R mAb bullous pemphigoid	

Removed from Phase I	Removed from Phase II	Removed from Phase III	Removed from registration
NME **AZD8154** Inhaled PI3Kgd asthma ***Calquence* + ceralasertib** BTK inhibitor + ATR inhibitor haematological malignancies **MEDI2228** BCMA antibody drug conjugate multiple myeloma	**NME** ***Lynparza*# + ceralasertib VIOLETTE** PARP inhibitor + ATR inhibitor breast cancer **MEDI6012** LCAT cardiovascular disease		

Phase progressions based on first patient dose achievement.
¶ Registrational Phase II/III trial # Partnered and/or in collaboration

Q1 2021 new molecular entity (NME)[1] pipeline

Phase I
19 New Molecular Entities

- AZD0466# BCL2/xL haematological and solid tumours
- AZD1390 glioblastoma
- AZD4573 CDK9 haematological malignancies
- AZD5305 PARP1Sel solid tumours
- AZD5991 MCL1 haematological malignancies
- AZD7648# DNAPK solid and haematological tumours
- AZD8701 FOXP3 solid tumours
- *Calquence* (platform) PRISM BTK + multiple novel onc therapies r/r aggressive NHL
- *Imfinzi*#+adavosertib# PD-L1+Wee1 solid tumours
- *Imfinzi*#+RT (platform) CLOVER PD-L1+RT HNSCC NSCLC SCLC
- *Imfinzi*#+tremelimumab PD-L1+CTLA-4 solid tumours
- *Imfinzi*#+tremelimumab+chemo PD-L1+CTLA-4 1L PDAC oesophageal SCLC
- *Imfinzi*+selumetinib# PD-L1+MEK solid tumours
- IPH5201# CD39 solid tumours
- MEDI1191 IL12 mRNA solid tumours
- MEDI5395 rNDV GMCSF solid tumours
- MEDI5752+Axitinib PD-1/CTLA-4+VEGF advanced renal cell carcinoma
- MEDI9253 rNDV IL12 solid tumours
- *Tagrisso* combo# TATTON EGFR+MEK/MET advanced EGFRm NSCLC

Phase II
21 New Molecular Entities

- adavosertib# Wee1 ovarian / uterine serous / solid tumours
- AZD2811 nanoparticle Aurora solid tumours, haematological malignancies
- camizestrant SERD ER+ breast
- capivasertib# AKT prostate
- ceralasertib ATR solid tumours
- *Imfinzi* (platform) HUDSON PD-L1+multiple novel ONC therapies post IO NSCLC
- *Imfinzi*# (platform) BALTIC PD-L1+CTLA-4, WEE1+Carboplatin, ATR+PARP ES-SCLC R/R
- *Imfinzi*# (platform) COAST PD-L1+multiple novel ONC therapies NSCLC
- *Imfinzi*# (platform) NeoCOAST PD-L1+multiple novel ONC therapies NSCLC
- *Imfinzi*# + imaradenant# + cabazitaxel PD-L1+A2aR+CTx prostate cancer
- *Imfinzi*#+*Lynparza*# ORION PD-L1+PARP 1L mNSCLC
- *Imfinzi*#+MEDI0457# PD-L1+DNA HPV vaccine HNSCC
- *Imfinzi*#+monalizumab# PD-L1+NKG2a solid tumours
- *Imfinzi*#+tremelimumab PD-L1+CTLA-4 biliary tract oesophageal
- *Imfinzi*#+tremelimumab PD-L1+CTLA-4 gastric cancer
- *Imfinzi*+FOLFOX+bevacizumab (platform) COLUMBIA1 PD-L1+chemo+VEGF+multiple novel ONC therapies 1L MSS-CRC
- *Imfinzi*+*Lynparza*# BAYOU PD-L1+PARP bladder
- MEDI5752 PD-1/CTLA-4 solid tumours
- oleclumab+chemo or *Imfinzi*#+oleclumab+chemo CD73+chemo or PDL1+CD73+chemo pancreatic
- Post-1L *Tagrisso* (platform) ORCHARD EGFR+multiple novel ONC therapies EGFRm NSCLC
- *Tagrisso*+savolitinib# SAVANNAH EGFR+MET advanced EGFRm NSCLC

Phase III
12 New Molecular Entities

- camizestrant+palbociclib SERENA-4 SERD+CDK4/6 1L HR+ HER2- breast cancer
- capivasertib#+abiraterone CAPItello-281 AKT+abiraterone PTEN deficient metastatic hormone sensitive prostate cancer
- capivasertib#+fulvestrant CAPItello-291 AKT+fulvestrant locally-advanced (inoperable) or metastatic breast
- capivasertib+chemotherapy CAPItello-290 AKT+chemotherapy mTNBC 1L
- datopotamab deruxtecan# TROPION-Lung01 TROP2 2L+ NSCLC without actionable mutation
- *Imfinzi*#+/-tremelimumab+chemo POSEIDON PD-L1+/-CTLA-4+SoC 1L NSCLC
- *Imfinzi*#+/-tremelimumab+CRT ADRIATIC PD-L1+/-CTLA-4+CRT LS-SCLC
- *Imfinzi*#+tremelimumab HIMALAYA PD-L1+CTLA-4 1L HCC
- *Imfinzi*#+tremelimumab+SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer
- *Lynparza*#+*Imfinzi*# DUO-E PARP+PD-L1 1L endometrial cancer
- *Lynparza*#+*Imfinzi*#+bevacizumab DUO-O PARP+PD-L1+VEGF 1L ovarian
- monalizumab#+cetuximab (INTERLINK-1) NKG2a+EGFR 2L+ relapsed metastatic HNSCC

Under Review
0 New Moelcular Entities

Phase progressions based on first patient dose achievement.
[1] Includes novel combinations and additional indications for assets where the lead is not yet launched.
Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

Oncology

Precision medicine approach being explored

Q1 2021 new molecular entity (NME)[1] pipeline

Phase I

13 New Molecular Entities

- AZD0284 RORg psoriasis / respiratory
- AZD0449 Inhaled JAK inhibitor asthma
- AZD1402# inhaled IL4Ra asthma
- AZD2373 Podocyte health nephropathy
- AZD2693 nonalcoholic steatohepatitis
- AZD3366 CD39L3 CV disease
- AZD3427 Relaxin ThP CV disease
- AZD4041# orexin 1 receptor antagonist opioid use disorder
- AZD9977 MCR cardiovascular
- MEDI0618# PAR2 antagonist mAb osteooarthritis pain
- MEDI1341# alpha synuclein parkinson's disease
- MEDI1814# amyloidβ alzheimer's disease
- MEDI8367 avb8 chronic kidney disease

Phase II

20 New Molecular Entities

- anifrolumab# Type I IFN receptor lupus nephritis
- anifrolumab# Type I IFN receptor SLE SC
- AZD4831 MPO HFpEF
- AZD5718 FLAP coronary artery disease / CKD
- AZD7986# DPP1 COPD
- AZD8233 hypercholesterolemia cardiovascular
- AZD8601# VEGF-A cardiovascular
- AZD9567 SGRM chronic inflammatory diseases
- brazikumab IL23 ulcerative colitis
- cotadutide GLP-1/glucagon T2D / obesity / NASH / DKD
- MEDI3506 IL33 diabetic kidney disease
- MEDI3506 IL33 AD / COPD / asthma / COVID-19
- MEDI5884# cholesterol modulation cardiovascular
- MEDI6570 LOX-1 CV disease
- MEDI7352 NGF/TNF OA pain / painful diabetic neuropathy
- navafenterol# MABA COPD
- suvratoxumab α-Toxin Staphylococcus pneumonia
- tezepelumab# TSLP atopic dermatitis
- tezepelumab# COURSE TSLP COPD
- verinurad URAT-1 CKD / HFpEF

Phase III

5 New Molecular Entities

- AZD7442 long-acting antibody combination COVID-19
- brazikumab¶ IL23 crohns disease
- nirsevimab# RSV mAb-YTE passive RSV immunisation
- PT027# ICS/SABA asthma
- tezepelumab# NAVIGATOR TSLP severe uncontrolled asthma

Under Review

1 New Molecular Entity

- anifrolumab# TULIP Type I IFN receptor SLE

Phase progressions based on first patient dose achievement.

[1] Includes novel combinations and additional indications for assets where the lead is not yet launched

Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

BioPharmaceuticals

Precision medicine approach being explored

Q1 2021 lifecycle management (LCM)[1] pipeline

Phase I
2 Projects

- *Enhertu*# (platform) DESTINY-Breast08 ADC breast
- *Imfinzi*#+azacitidine# PD-L1+azacitidine MDS

Phase II
9 Projects

- *Enhertu*# (platform) DESTINY-Breast07 ADC breast
- *Enhertu*# DESTINY-CRC-01 ADC colorectal cancer
- *Enhertu*# DESTINY-Gastric02 ADC gastric
- *Enhertu*# DESTINY-Lung01 ADC NSCLC
- *Enhertu*# DESTINY-PanTumor01 HER2 targeting ADC HER2-expressing solid tumours
- *Enhertu*# DESTINY-PanTumor02 HER2 targeting ADC HER2-expressing solid tumours
- *Imfinzi*# (platform) BEGONIA PD-L1 1L mTNBC
- *Imfinzi*# (platform) MAGELLAN PD-L1 1L mNSCLC
- *Lynparza*# (basket) MK-7339-002 / LYNK002 PARP HRRm cancer

Phase III
30 Projects

- *Calquence*# BTK inhibitor 1st line MCL
- *Calquence*# BTK inhibitor r/r CLL, high risk
- *Calquence*#+venetoclax+ obinutuzumab BTK+BCL-2+anti-CD20 1st line CLL
- *Calquence*+R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL
- *Enhertu*# DESTINY-Breast02 ADC breast
- *Enhertu*# DESTINY-Breast03 ADC breast
- *Enhertu*# DESTINY-Breast04 ADC breast
- *Enhertu*# DESTINY-Breast05 ADC breast
- *Enhertu*# DESTINY-Breast06 ADC breast
- *Imfinzi*# + FLOT MATTERHORN PD-L1+CTx neo-adjuvant/adjuvant gastic cancer
- *Imfinzi*# CALLA PD-L1 adj. locally-advanced cervical cancer
- *Imfinzi*# PEARL PD-L1 1L metastatic NSCLC
- *Imfinzi*# post-SBRT PACIFIC-4 PD-L1 post-SBRT stage I/II NSCLC
- *Imfinzi*# POTOMAC PD-L1 non muscle invasive bladder cancer
- *Imfinzi*#+CRT KUNLUN PD-L1+CRT locally-advanced esophageal squamous cell carcinoma
- *Imfinzi*#+CRT PACIFIC-2 PD-L1+CRT NSCLC
- *Imfinzi*#+CRT PACIFIC-5 (China) PD-L1+CRT locally-advanced stage III NSCLC
- *Imfinzi*#+Ctx MERMAID-1 PD-L1 stage II-III adjuvant NSCLC
- *Imfinzi*#+CTx neoadjuvant AEGEAN PD-L1+CTx locally-advanced stage II-III NSCLC
- *Imfinzi*#+CTx NIAGARA PD-L1+CTx muscle invasive bladder cancer
- *Imfinzi*#+CTx TOPAZ-1 PD-L1+CTx 1L biliary tract cancer
- *Imfinzi*#+VEGF EMERALD-2 PD-L1+VEGF adjuvant HCC
- *Imfinzi*#+VEGF+TACE EMERALD-1 PD-L1+VEGF+TACE locoregional HCC
- *Lynparza*# LYNK-003 PARP platinum sensitive 1L colorectal cancer
- *Lynparza*# OlympiA PARP gBRCA adjuvant breast
- *Lynparza*# SOLO-3 PARP BRCAm PSR ovarian
- *Lynparza*+abiraterone# PROpel PARP+NHA prostate cancer
- *Tagrisso* +/- CTx neoadjuvant NeoADAURA EGFR stage II/III resectable EGFRm
- *Tagrisso* LAURA EGFRm locally-advanced unresectable NSCLC
- *Tagrisso*+chemo FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC

Under Review
0 Projects

Phase progressions based on first patient dose achievement.
[1] Includes significant LCM projects and parallel indications for assets beyond Phase III
Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

Oncology

Precision medicine approach being explored

Q1 2021 lifecycle management (LCM)[1] pipeline

Phase I
0 Projects

Phase II
3 Projects

- *Fasenra* ARROYO
  IL5R chronic spontaneous urticaria
- *Fasenra* HILLIER
  IL5R atopic dermatitis
- roxadustat#
  HIF-PH inhibitor chemo induced anaemia

Phase III
10 Projects

- *Breztri*
  LABA/LAMA/ICS asthma
- *Farxiga/Forxiga* DAPA-MI
  SGLT2 prevention of HF and CV death following a myocardial infarction
- *Farxiga/Forxiga* DELIVER
  SGLT2 HFpEF
- *Fasenra* FJORD
  IL5R bullous pemphigoid
- *Fasenra* MANDARA
  IL5R EGPA
- *Fasenra* MESSINA
  IL5R eosinophilic esophagitis
- *Fasenra* NATRON
  IL5R hypereosinophilic syndrome
- *Fasenra*# OSTRO, ORCHID
  IL5R nasal polyps
- *Fasenra*# RESOLUTE
  IL5R COPD
- roxadustat#
  HIFPH anaemia MDS

Under Review
1 Project

- *Farxiga/Forxiga* DAPA-CKD
  SGLT2 CKD

Phase progressions based on first patient dose achievement.
[1] Includes significant LCM projects and parallel indications for assets beyond Phase III
Partnered and/or in collaboration; ¶ Registrational Phase II/III trial

BioPharmaceuticals

Precision medicine approach being explored

Estimated key regulatory submission acceptances

	H1 2021	H2 2021	2022	2022+	2022+	2022+
NME	AZD7442 SARS-CoV-2		nirsevimab passive RSV immunisation	camizestrant + palbociclib breast cancer SERENA-4	datopotamab deruxtecan NSCLC TROPION-Lung01	
			PT027 asthma	capivasertib + fulvestrant locally advanced or mBC CAPItello-291	*Imfinzi* + tremelimumab + SoC urothelial NILE	
	COVID-19 Vaccine AstraZeneca SARS-CoV-2 (US, JP)	*Imfinzi* + tremelimumab HCC HIMALAYA	*Imfinzi* + tremelimumab + CRT LDS-SCLC ADRIATIC	capivasertb + CTx 1L mTNBC CAPItello-290	*Lynparza* + *Imfinzi* endometrial cancer DUO-E	brazikumab crohns disease
	tezepelumab asthma NAVIGATOR	*Imfinzi* +/- tremelimumab NSCLC POSEIDON	*Koselugo* NF1 (China / Japan) SPRINT	capivasertb + abiraterone PTEN deficient mHSPC CAPItello-281	*Lynparza* + *Imfinzi* + bevacizumab ovarian DUO-O	*Fasenra* severe asthma (China)
LCM	*Calquence* r/r CLL, high risk ELEVATE-RR	*Enhertu* DESTINY-Breast03	*Calquence* 1L CLL ELEVATE-TN (China)	*Calquence* + R-CHOP 1L DLBCL ESCALADE	*Imfinzi* post-SBRT NSCLC PACIFIC-4	*Breztri/Trixeo* asthma KALOS, LOGOS
	Fasenra nasal polyps OSTRO	*Imfinzi* + CRT NSCLC PACIFIC-2	*Enhertu* DESTINY-Breast02	*Calquence* + venetoclax + obinutuzumab 1L CLL AMPLIFY	*Imfinzi* cervical CALLA	*Farxiga* prevention of HF and CV death following a myocardial infarction DAPA-MI
		Lynparza breast OLYMPIA	*Enhertu* DESTINY-Breast04	*Calquence* 1L MCL ECHO	*Imfinzi* adjuvant NSCLC BR.31	*Fasenra* COPD RESOLUTE
		Lynparza + abiraterone prostate PROPEL	*Imfinzi* NSCLC PEARL	*Enhertu* DESTINY-Breast05	*Imfinzi* non muscle invasive bladder POTOMAC	*Fasenra* EGPA MANDARA
			Imfinzi + CTx biliary tract TOPAZ-1	*Enhertu* DESTINY-Breast06	*Lynparza* platinum sensitive 1L colorectal LYNK-003	*Fasenra* HES NATRON
			Imfinzi + VEGF + TACE locoregional HCC EMERALD-1	*Imfinzi* + CRT LA ESCC KUNLUN	monalizumab + cetuximab 2L+ relapsed metastatic HNSCC INTERLINK-1	*Fasenra* nasal polyps ORCHID (China / Japan)
			Lynparza ovarian SOLO-3	*Imfinzi* + CRT NSCLC PACIFIC-5 (China)	*Tagrisso* stage II/III resectable EGFRm NSCLC NeoADAURA	*Fasenra* bullous pemphigoid FJORD
			Duaklir Genuair COPD (China)	*Imfinzi* neoadjuvant NSCLC AEGEAN	*Tagrisso* locally adv. unresectable NSCLC LAURA	tezepelumab asthma NAVIGATOR
			Farxiga HF (HFpEF) DELIVER	*Imfinzi* + CRT neo-adjuvant/adjuvant gastric MATTERHORN	*Tagrisso* + CTx EGFRm NSCLC FLAURA2	
			Fasenra eosinophilic esophagitis MESSINA	*Imfinzi* + CTx stage II-III adjuvant NSCLC MERMAID-1	*Imfinzi* + chemo muscle invasive bladder NIAGARA	
			roxadustat anemia in MDS	*Imfinzi* + VEGF adjuvant HCC EMERALD-2		
			Xigduo XR/Xigduo type-2 diabetes (China)			

Oncology BioPharmaceuticals

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

Designations

5 Accelerated approvals

- *Lynparza* ovarian cancer SOLO-2 (US)
- *Tagrisso* EGFRm T790M NSCLC (US)
- *Imfinzi* bladder cancer (US)
- *Calquence* MCL (US)
- *Enhertu* unresectable or HER2+ MBC 3L DESTINY-Breast01 (US)

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

- *Tagrisso* EGFRm T790M NSCLC (US)
- *Lynparza* prostate cancer PROFOUND (US)
- *Imfinzi* bladder cancer 1L (US)
- *Calquence* MCL (US)
- *Imfinzi* stage III NSCLC 1L PACIFIC (US)
- *Tagrisso* NSCLC 1L FLAURA (US)
- tezepelumab asthma (US)
- nirsevimab RSV mAB (US)
- nirsevimab RSV mAB (EU)[1]
- selumetinib NFI type 1 SPRINT (US)
- *Enhertu* DESINTY-BREAST01 (US)
- *Calquence* CLL (US)
- *Enhertu* gastric cancer (JP)[2]
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
- *Enhertu* HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
- *Tagrisso* adjuvant NSCLC ADAURA (US)
- *Forxiga* CKD DAPA-CKD (US)

12 Fast Track

- MEDI3902 Psl-PcrV pneumo Px (US)
- savratoxumab Staph HAP (US)
- *Imfinzi* NSCLC (US)
- nirsevimab (MEDI8897) RSV mAB (US)
- *Imfinzi* HNSCC HAWK (US)
- anifrolumab SLE (US)
- *Lynparza* ovarian cancer SOLO-2 (US)
- *Tagrisso* EGFRm T790M NSCLC (CN)
- *Farxiga* HFrEF (US)
- *Farxiga* chronic kidney disease (US)
- cotadutide non-alcoholic steatohepatitis (US)
- *Farxiga* MI RRCT DAPA-MI (US)

36 Priority Review / RTOR[3]

- *Tagrisso* EGFRm T790M NSCLC (JP)
- *Tagrisso* EGFRm T790M NSCLC (US)
- *Imfinzi* bladder cancer 2L (US)
- *Tagrisso* NSCLC AURA3 (US)
- *Calquence* MCL (US)
- *Lynparza* breast cancer OLYMPIAD (US)
- roxadustat CKD (CN)
- *Tagrisso* NSCLC FLAURA (US)
- *Imfinzi* stage III NSCLC PACIFIC (EU)
- *Imfinzi* stage III NSCLC PACIFIC (JP)
- *Lynparza* tablet (US)
- *Lynparza* tablet (CN)
- *Lynparza* breast cancer OLYMPIAD (JP)
- *Tagrisso* NSCLC 1L FLAURA (JP)
- *Lumoxiti* HCL PLAIT (US)
- *Lynparza* ovarian SOLO-1 (US)
- *Lynparza* ovarian SOLO-1 (CN)
- *Breztri Aerosphere* (PT010) COPD (CN)
- *Tagrisso* NSCLC 1L FLAURA (CN)
- *Breztri Aerosphere* (PT010) (CN)
- *Lokelma* hyperkalaemia (CN)
- *Lynparza* pancreatic 1L (US)
- *Enhertu* DESINTY-BREAST01 (US)
- *Farxiga* HF DAPA-HF (US)
- *Imfinzi* +/-treme+SOC SCLC 1L CASPIAN (US)
- *Lynparza* prostate PROfound (US)
- *Lynparza* +Avastin ovarian 1L PAOLA-1 (US)
- *Koselugo*/selumetinib NFI type 1 SPRINT (US)
- *Calquence* CLL ELEVATE-TN, ASCEND[3] (US)
- *Brilinta* stroke THALES (US)
- *Imfinzi* Q4W regimen NSCLC, bladder (US)
- *Tagrisso* adjuvant NSCLC ADAURA (US)
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
- *Lynparza* prostate PROfound (CN)
- *Forxiga* CKD DAPA-CKD (US)
- *Forxiga* CKD DAPA-CKD (JP)

29 Orphan

- *Lynparza* ovarian cancer SOLO-2 (US)
- *Lumoxiti* HCL PLAIT (US)
- *Lumoxiti* HCL PLAIT (EU)
- *Crestor* paediatric (US)
- cediranib VEGFR tki (US)
- *Iressa* EGFRm NSCLC (US)
- *Tagrisso* EGFRm T790M NSCLC (US)
- AZD3241 MPO (EU)
- *Calquence* CLL 1L (US)
- *Calquence* MCL (US)
- *Calquence* WM (US)
- *Calquence* CLL 1L (EU)
- selumetinib thyroid cancer ASTRA (US)
- *Lynparza* breast cancer OLYMPIAD (JP)
- *Lynparza* ovarian cancer SOLO-2 (JP)
- *Koselugo*/selumetinib NFI type 1 SPRINT (US)
- *Koselugo*/selumetinib NFI type 1 SPRINT (EU)
- *Lynparza* pancreatic cancer POLO (US)
- *Fasenra* EGPA (US)
- *Fasenra* HES (US)
- saracatinib IPF (US)
- *Imfinzi* +/-treme+SOC SCLC 1L CASPIAN (US)
- *Fasenra* EoE (US)
- *Imfinzi* +treme HCC 1L HIMALAYA (US)
- *Lynparza* pancreatic cancer POLO (JP)
- *Enhertu* HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
- *Koselugo* /selumetinib NFI type 1 SPRINT (JP)
- *Imfinzi+CTx* biliary tract 1L TOPAZ-1 (US)
- Imfinz+/- tremelimumab HCC 1L HIMALAYA (EU)

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

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

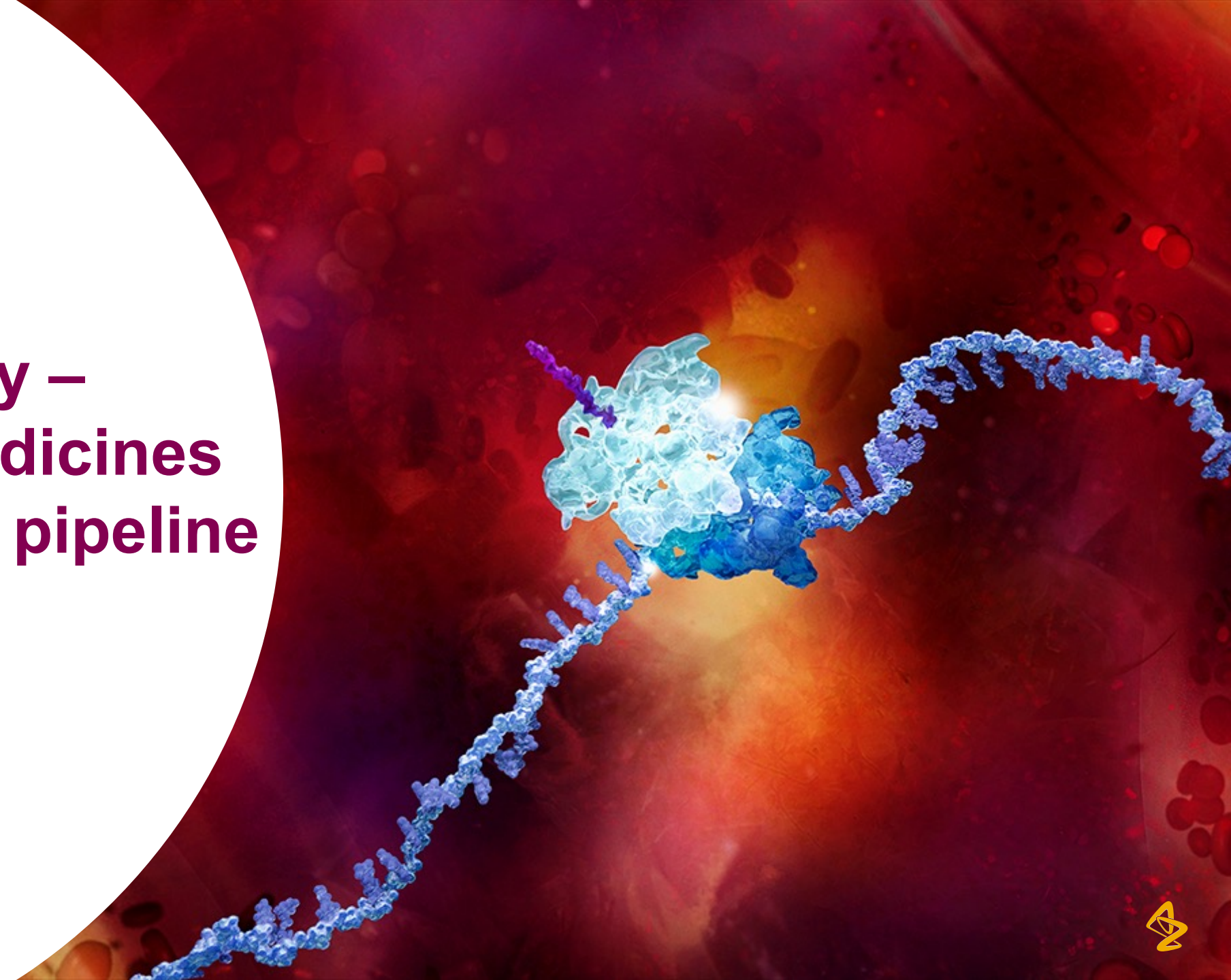
FAST TRACK is a process designed to facilitate the development, and expedite the review of medicines to treat serious conditions and fill an unmet medical need. [3]REAL-TIME ONCOLOGY REVIEW (RTOR) and Project Orbis is an initiative of the FDA Oncology Centre of Excellence (OCE) providing a framework for concurrent submission and review of oncology products among international partners.

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

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

Oncology BioPharmaceuticals

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: 2022+
Phase III ASTRIS NCT02474355	Real world setting in adult patients with advanced or metastatic, EGFRm T790M+ NSCLC	3,020	Single-arm trial - *Tagrisso* Global trial - 16 countries	• Primary endpoints: OS and safety • Secondary endpoint: PFS	• FPCD: Q3 2015 • LPCD: Q4 2017
Phase II ELIOS NCT03239340	EGFR TKI treatment-naïve patients with locally-advanced or metastatic EGFRm NSCLC	150	Single arm trial - *Tagrisso* Global trial - five countries	• Primary Endpoint: proportion of patients with a given tumour genetic and proteomic marker at the point of disease progression as defined by the investigator • Secondary endpoint: PFS, ORR, DoR	• FPCD: Q2 2018
Phase I ODIN-BM NCT03463525	Patients with EGFRm NSCLC with brain metastases	8	Single-arm trial - *Tagrisso*	• Primary Endpoints: assessments of brain standard uptake value (SUV) and pharmacokinetics (PK) • Secondary endpoints: PK	• FPCD: Q4 2018 • LPCD: Q1 2020 • Data anticipated: H2 2021

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 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: 2022+
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: 2022+
Phase II **ORCHARD** **NCT03944772**	Advanced EGFRm NSCLC patients who have progressed on first line *Tagrisso* treatment	182	Modular design platform trial: • Module 1: *Tagrisso* + savolitinib • Module 2: *Tagrisso* + gefitinib • Module 3: *Tagrisso* + necitumumab • Module 4: *carboplatin + pemetrexed + Imfinzi* • *Module 5: Tagrisso + alectinib* • *Module 6: Tagrisso + selpercatinib* • No intervention: observational cohort Global trial - 8 countries	• Primary endpoint: ORR • Secondary endpoints: PFS, DoR, OS, safety and tolerability	• FPCD: Q3 2019 • Data anticipated: 2022+
Phase II **SAVANNAH** **NCT03778229**	EGFRm / MET+, locally advanced or metastatic NSCLC who have progressed following treatment with *Tagrisso*	259	• Single arm trial: *Tagrisso* + savolitinib Global trial	• Primary endpoint: ORR • Secondary endpoints include PFS, DoR and OS	• FPCD Q1 2019 • Data anticipated: 2022+
Phase Ib **TATTON** **NCT02143466**	Advanced EGFRm NSCLC TKI failure	344	• Arm 1: *Tagrisso* + *Imfinzi* • Arm 2: *Tagrisso* + savolitinib • Arm 3: *Tagrisso* + selumetinib Enrolment to *Tagrisso* + *Imfinzi* arm will not restart Global trial	• Safety, tolerability, pharmacokinetics and preliminary anti-tumour activity	• FPCD: Q3 2014 • Data readout: H2 2020

Imfinzi (PD-L1 mAb)

NSCLC, early disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III MERMAID-1 NCT04385368	Completely resected Stage II and III NSCLC	332	• Arm 1: *Imfinzi* + SoC chemo • Arm 2: placebo + SoC chemo	Primary endpoint: • DFS Secondary endpoint • DFS, OS,	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase III MERMAID-2 NCT04642469	Completely resected Stage II-III NSCLC	284	• Arm 1: *Imfinzi* • Arm 2: placebo	Primary endpoint: • DFS Secondary endpoint • DFS, PFS, OS	• FPCD: Q4 2020 • Data anticipated: 2022+
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 endpoint: • mPR, EFS Secondary endpoint • pCR	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase III ADJUVANT BR.31 NCT02273375 Partnered	Adjuvant NSCLC patients Ib (≥4cm) – stage IIIa resected NSCLC (incl. EGFR/ALK positive)	1,360	• 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: 2022+
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 endpoint: • OS, ORR	• FPCD: Q2 2018 • LPCD: Q3 2019 • Data anticipated: H2 2021
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: 2022+
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: 2022+
Phase II/III Lung Master Protocol NCT02154490 Partnered	Stage IV squamous NSCLC patients Biomarker-targeted 2L therapy	140	• Subtrial A: *Imfinzi* (non-match for other biomarker driven subtrials) i.v. Q2W single arm *Imfinzi* Phase II only • Subtrial B: PI3K inhibitor vs. docetaxel • Subtrial C: CDK4/6 inhibitor vs, docetaxel • Subtrial D: AZD4547 (FGFR inhibitor) vs. docetaxel • Subtrial E: C-MET/HGFR Inhibitor + erlotinib vs. erlotinib	Primary endpoints: • ORR • PFS • OS	• FPCD: Q2 2014 • Data anticipated: 2022+

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

Lung cancer, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
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 2021
Phase III **POSEIDON** **NCT03164616**	NSCLC 1L	1,000	• Arm 1: *Imfinzi* + chemo • Arm 2: *Imfinzi* + tremelimumab + chemo • Arm 3: SoC	Primary endpoint: • OS • PFS	• FPCD: Q2 2017 • LPCD: Q4 2018 • Data readout: Q4 2019 • PFS primary endpoint met • OS data anticipated: H1 2021
Phase II **MAGELLAN** **NCT03819465**	NSCLC 1L	212	• Arm A1: *Imfinzi* • Arm A2: *Imfinzi* + danvatirsen • Arm A3: *Imfinzi* + oleclumab • Arm A3: MEDI5752 • 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	Primary endpoint: • Safety & tolerability Secondary endpoint: • ORR, DoR, PFS, OS, PK, ADA	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase III **ADRIATIC** **NCT03703297**	Limited stage SCLC 1L following platinum-based concurrent chemoradiation therapy	600	• Arm 1: *Imfinzi* + tremelimumab (4 doses) • Arm 2: *Imfinzi* • Arm 3: placebo	Primary endpoints: • PFS • OS	• FPCD: Q4 2018 • Data anticipated: 2022
Phase III **CASPIAN** **NCT03043872**	Extensive stage SCLC 1L	805	• Arm 1: *Imfinzi* + tremelimumab + EP (carboplatin or cisplatin + etoposide) • Arm 2: *Imfinzi* + EP (carboplatin or cisplatin + etoposide) • Arm 3: EP (carboplatin or cisplatin + etoposide)	Primary endpoint: • OS	• FPCD: Q1 2017 • LPCD: Q2 2018 • Data readout: Q2 2019 • OS Primary endpoint met for *Imfinzi* monotherapy • OS primary endpoint not met for *Imfinzi* + tremelimumab
Phase II **BALTIC** **NCT02937818**	SCLC	72	• Arm A: *Imfinzi* + tremelimumab Q4W • Arm B: adavosertib and carboplatin BID • Arm C: ceralasertib and *Lynparza*	• Primary endpoint: ORR	• FPCD: Q4 2016 • Data anticipated: H1 2021

Imfinzi (PD-L1 mAb)

Other cancers, early disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III POTOMAC NCT03528694	Non-muscle invasive bladder cancer	975	• Arm 1: BCG (Induction + maintenance) • Arm 2: *Imfinzi* + BCG (Induction only) • Arm 3: *Imfinzi* + BCG (Induction + maintenance)	Primary endpoints: • DFS	• FPCD: Q2 2018 • LPCD: Q4 2020 • Data anticipated: 2022+
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	1,050	• Arm 1: *Imfinzi* in combination with gemcitabine + cisplatin, *Imfinzi* maintenance • Arm 2: gemcitabine + cisplatin	Coprimary endpoints: • pCR • EFS	• FPCD: Q4 2018 • Data anticipated: 2022+
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 1 vs Arm 3 Secondary endpoint PFS for Arm 2 vs Arm 3 , OS	• FPCD: Q1 2019 • Data anticipated: 2022
Phase III EMERALD-2 NCT03847428	Adjuvant therapy in HCC	888	• Arm 1: *Imfinzi* + bevacizumab • Arm 2: *Imfinzi* + placebo • Arm 3: placebo + placebo	Primary endpoint: • RFS for Arm 2 vs Arm 3 Secondary endpoint: • RFS Arm 1 vs Arm 3, OS, RFS at 24m	• FPCD: Q2 2019 • Data anticipated: 2022+
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: 2022+
Phase III MATTERHORN NCT04592913	Resectable GC/GEJC	900	• Arm 1: *Imfinzi* + FLOT • Arm 2: placebo + FLOT	Primary endpoint: • EFS Secondary endpoint: • OS Arm 1 vs Arm 2 • pCR Arm 1 vs Arm 2	• FPCD: Q4 2020 • Data anticipated: 2022+

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

Other cancers, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **NILE** **NCT03682068**	Bladder cancer 1L	1,215	• Arm 1: *Imfinzi* + tremelimumab + SoC • Arm 2: *Imfinzi* + SoC • Arm 3: SoC	Primary endpoint: • OS	• FPCD: Q4 2018 • Data anticipated: 2022+
Phase III **KESTREL** **NCT02551159**	HNSCC 1L	823	• Arm 1: *Imfinzi* • Arm 2: *Imfinzi* + tremelimumab • Arm 3: SoC	Primary endpoint: • OS Secondary endpoint: • PFS, ORR, DoR, safety, biomarkers	• FPCD: Q4 2015 • LPCD: Q1 2017 • Data readout: Q1 2021 • Primary endpoint not met
Phase III **HIMALAYA** **NCT03298451**	HCC 1L	1,324	• Arm 1: *Imfinzi* + tremelimumab • Arm 2: *Imfinzi* • Arm 3: sorafenib	Primary endpoint: • OS Secondary endpoint: • PFS, TTP, ORR	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data anticipated: H2 2021
Phase II **NCT02527434**	Urothelial bladder cancer triple-negative breast cancer pancreatic ductal-adenocarcinoma	76	• Arm 1 tremelimumab (urothelial bladder cancer) • Arm 2 tremelimumab (triple-negative breast cancer) • Arm 3 tremelimumab (pancreatic ductal-adenocarcinoma)	Primary endpoint: • ORR Secondary endpoints: • Safety, DoR	• FPCD: Q4 2015 • LPCD: Q4 2016 • Data readout: Q4 2018
Phase III **TOPAZ-1** **NCT03875235**	BTC 1L	757	• Treatment Arm 1 *Imfinzi* + gemcitabine + cisplatin • Treatment Arm 2 placebo + gemcitabine + cisplatin Global trial	Primary endpoint: • OS Secondary endpoint: • PFS, ORR, DoR	• FPCD Q2 2019 • LPCD: Q4 2020 • Data anticipated: 2022
Phase III **CALLA** **NCT03830866**	Locally advanced cervical cancer	714	• Arm 1 *Imfinzi* + EBRT + brachytherapy with platinum • Arm 2 placebo + EBRT + brachytherapy with platinum Global trial	Primary • PFS Secondary • OS, CR rate, DoR, ORR, safety/tolerability	• FPCD: Q1 2019 • LPCD: Q4 2020 • Data anticipated: 2022+

Imfinzi (PD-L1 mAb) +/- treme (CTLA-4 mAb)

Other cancers, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **STRONG** **NCT03084471**	Advanced solid malignancies	1,200	• Arm 1: *Imfinzi* • Arm 2: *Imfinzi* + tremelimumab	• Primary endpoint: safety	• FPCD: Q2 2017 • Data anticipated: 2022+
Phase I **NCT02658214**	Solid tumours	80	• Arm 2 SCLC: *Imfinzi* + tremelimumab + carboplatin + etoposide • Arm 3 TNBC: *Imfinzi* + tremelimumab + chemo • Arm 4 TNBC: *Imfinzi* + tremelimumab + chemo • Arm 5 GEJ: *Imfinzi* + tremelimumab + oxaliplatin + 5-FU + leucovorin • Arm 6 PDAC: *Imfinzi* + tremelimumab + chemo • Arm 7 ESSC: *Imfinzi* + tremelimumab + chemo	• Safety	• FPCD: Q1 2016 • LPCD: Q1 2019 • Data anticipated: 2022+
Phase I **CLOVER** **NCT03509012**	HNSCC, NSCLC, SCLC	102	• HNSCC Arm 1 • NSCLC Arm 1 • NSCLC Arm 2 • NSCLC Arm 3 • SCLC Arm 2 • SCLC Arm 3 • SCLC Arm 4	• Safety	• FPCD: Q2 2018 • Data anticipated: H1 2021
Phase II **BEGONIA** **NCT03742102**	mTNBC 1L	220	• Arm 1 *Imfinzi* + paclitaxel • Arm 2 *Imfinzi* + paclitaxel + capivasertib • Arm 4 *Imfinzi* + paclitaxel + danvatirsen • Arm 5 *Imfinzi* + paclitaxel + oleclumab • Arm 6 *Imfinzi* + *Enhertu* • Arm 7 *Imfinizi* + datopotamab deruxtecan Global trial	Primary endpoint: • Safety and tolerability Secondary endpoint: • ORR, PFS, DoR, OS, PK, ADA	• FPCD: Q1 2019 • Data anticipated: 2022+

Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered	*BRCA*m adjuvant breast cancer	1,836	• Arm 1: *Lynparza* BiD 12 month duration • Arm 2: placebo 12-month duration Global trial partnership with BIG and NCI/NRG	• 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 PROfound NCT02987543	Metastatic castration-resistant prostate cancer HRRm, 2L+	387	• Arm 1: *Lynparza* BID • Arm 2: physician's choice: enzalutamide 160mg once daily or abiraterone acetate 1,000mg once daily Global trial	• Primary endpoint: radiologic PFS • Secondary endpoints: ORR, Time to Pain Progression, OS	• FPCD: Q2 2017 • LPCD: Q4 2018 • Data readout : Q3 2019 • Primary endpoint met

Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III **DuO-O** **NCT03737643**	Advanced ovarian cancer 1L	1,256	Non t*BRCA*m (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: 2022+
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: 2022+
Phase II **ORION** **NCT03775486**	Stage IV NSCLC whose disease has not progressed following SoC chemo + *Imfinzi* Maintenance therapy 1L	250	• Arm 1: *Imfinzi* + *Lynparza* • Arm 2: *Imfinzi* + placebo Global trial	Primary endpoint: • PFS Secondary enpoints: • OS, ORR, DoR, PFS in HRRm, PK, ADA	• FPCD Q1 2019 • Data anticipated: H1 2021
Phase II **BAYOU** **NCT03459846**	Platinum-Ineligible unresectable Stage IV urothelial cancer	154	• Arm 1: *Imfinzi* + *Lynparza* • Arm 2: *Imfinzi* + placebo Global trial	• Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR, PFS in HRRm, PFS6, PK, ADA, PRO	• FPCD: Q2 2018 • LPCD: Q3 2019 • Data anticipated : H1 2021
Phase I / II **MEDIOLA** **NCT02734004**	g*BRCA*m ovarian cancer 2L+ g*BRCA*m HER2-negative breast cancer 1-3L SCLC 2L+ Gastric cancer 2L+	148	• Arm 1: *Lynparza* + *Imfinzi* • Dose until progression Global trial	Primary endpoints: • DCR at 12 weeks • Safety and tolerability	• FPCD: Q2 2016 • LPCD: Q2 2017
Phase I / II **MEDIOLA** **(Ovarian expansion)** **NCT02734004**	g*BRCA*m ovarian cancer 2L+ Non-g*BRCA*m ovarian cancer 2L+ Non-g*BRCA*m ovarian cancer 2L+	115	• Arm 1: *Lynparza* + *Imfinzi* • Arm 2: *Lynparza* + *Imfinzi* • Arm 3: *Lynparza* + *Imfinzi* + bevacizumab • Dose until progression Global trial	Primary endpoints: • DCR at 12 weeks • ORR • Safety and tolerability	• FPCD: Q2 2018 • LPCD: Q2 2020

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	Primary Endpoint: • rPFS Secondary endpoints: • TFST, TTPP, OS	• FPCD: Q4 2018 • Data anticipated: H2 2021
Phase II/III **GY005** **NCT02502266** **Externally sponsored**	Recurrent platinum resistant/refractory ovarian cancer	680	• Arm 1: chemo • Arm 2: cediranib + *Lynparza* • Arm 3: cediranib • Arm 4: *Lynparza* US/Canada sites	Primary endpoints: • PFS, OS Secondary endpoints: • ORR, QoL, safety	• FPCD: Q2 2016 • Data anticipated: 2022+
Phase II **LYNK-002** **NCT03742895** **Partnered**	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
Phase III **LYNK-003** **NCT04456699** **Partnered**	Advanced colorectal cancer 1L maintanence	525	• Arm 1: bevacizumab + 5-FU maintenance • Arm 2: bevacizumab + *Lynparza* maintenance • Arm 3: *Lynparza* maintenance Global trial	Primary endpoints: • PFS Secondary endpoints: • OS, ORR, DoR, AEs	• FPCD: Q3 2020 • Data anticipated: 2022+
Phase II **DUETTE** **NCT04239014**	Ovarian post-PARPi maintenance PSR	192	• Arm 1: *Lynparza* + ceralasertib • Arm 2: *Lynparza* • Arm 3: placebo Global trial	Primary endpoint • PFS Secondary endpoints • TTSP, ORR, OS • Safety and tolerability	• Initiating • Data anticipated: 2022+

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **DESTINY-Breast01** **NCT03248492**	HER2-positive, unresectable and/or metastatic breast cancer patients previously treated with trastuzumab emtansine	230	Randomised, open label, sequential assignment • *Enhertu*	Primary endpoint ORR Secondary end points DoR, CBR, PFS, OS	• FPCD: Q4 2017 • LPCD: Q4 2018 • Data readout: Q2 2019 • Primary objective met
Phase III **DESTINY-Breast02** **NCT03523585**	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* Physicians choice of lapatinib + capecitabine or trastuzumab + capecitabine	Primacy endpoint PFS Secondary endpoints OS, ORR, DoR, CBR	• FPCD: Q4 2018 • LPCD: Q4 20202 • Data anticipated: 2022
Phase III **DESTINY-Breast03** **NCT03529110**	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: Q4 2018 • LPCD: Q2 2020 • Data anticipated: H2 2021
Phase III **DESTINY-Breast04** **NCT03734029**	HER2-low, unresectable and/or metastatic breast cancer patients	540	Randomised open label parallel assignment • *Enhertu* • Physicians choice of SoC chemo (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel)	Primary end point PFS Secondary end points OS, DoR, ORR	• FPCD: Q4 2018 • LPCD: Q4 2020 • Data anticipated: 2022
Phase III **DESTINY-Breast05** **NCT04622319**	High-risk HER2-positive patients with residual invasive breast cancer following neoadjuvant therapy	1,600	Randomised open label parallel assignment • *Enhertu* • Ado-trastuzumab emtansine	Primary end point IDFS Secondary end points DFS, OS, DRFI, BMFI	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase III **DESTINY-Breast06** **NCT04494425**	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 end point PFS Secondary end points OS, DoR, ORR	• FPCD Q2 2020 • Data anticipated: 2022+
Phase Ib/II **DESTINY-Breast07** **NCT04538742**	HER2-positive metastatic breast cancer	350	Randomised open label sequential assignment • *Enhertu* • *Enhertu* + *Imfinzi* • *Enhertu* + pertuzumab • *Enhertu* + paclitaxel • *Enhertu* + *Imfinzi* + paclitaxel • *Enhertu* + tucatinib	Primary end point AE, SAE Secondary end points ORR, PFS, DoR, OS	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase Ib **DESTINY-Breast08** **NCT04556773**	HER2-low metastatic breast cancer	185	Non-Randomised open label parallel assignment • *Enhertu* + capecitabine • *Enhertu* + *Imfinzi* + paclitaxel • *Enhertu* + capivasertib • *Enhertu* + anastrozole • *Enhertu* + *Faslodex*	Primary end point AE, SAE Secondary end points ORR, PFS, DoR, OS	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase III **DESTINY-Breast09** **NCT04784715**	HER2-positive, metastatic breast cancer, no prior therapy for advanced or metastatic disease	1134	Randomized, parallel assignment • *Enhertu + placebo* • *Enhertu + pertuzumab* • *Standard of Care*	Primary end point PFS Secondary end points OS, DoR, ORR	• Initiating

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Gastric cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase II **DESTINY-Gastric01** **NCT03329690**	HER2-overexpressing advanced gastric or gastroesophogeal junction adenocarcinoma patients who have progressed on two prior treatment regimens	233	Randomised open label parallel assignment • *Enhertu* • SoC chemo Trial conducted in Japan and Korea	Primary end point ORR Secondary end points 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**	HER2-positive gastric cancer that cannot be surgically removed or has spread	79	Open label single group assignment • *Enhertu* Trial conducted in Western population	Primary endpoint ORR Secondary endpoints PFS, ORR, OS, DoR	• FPCD: Q4 2019 • Data anticipated: H2 2021
Phase Ib/II **DESTINY-Gastric03** **NCT04379596**	HER2-overexpressing gastric or gastroesophogeal junction cancer patients	250	• 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 durvalumab Global trial 8 countries	Part 1 Primary endpoint safety Part 2 Primary endpoint ORR Secondary end points DoR, DCR, PFS, OS, range of PK endpoints, ADAs	• FPCD: Q2 2020
Phase III **DESTINY-Gastric04** **NCT04704934**	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	• Initiating

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **DESTINY-Lung01** **NCT03505710**	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	• FPCD: Q2 2018 • Data anticipated: H2 2021
Phase II **DESTINY-Lung02** **NCT04644237**	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
Phase Ib **DESTINY-Lung03** **NCT04686305**	HER2-over-expressing, unresectable and/or metastatic NSCLC	120	Non *randomised parallel group assignment* • *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	• Initiating

Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase II **DESTINY-PanTumour02** **NCT04482309**	HER2 expressing tumours	280	Non randomised single group assignment • *Enhertu*	Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS	• FPCD: Q4 2020
Phase II **DESTINY-PanTumour01** **NCT04639219**	HER2m expressing tumours	100	Non-randomised single group assignment • *Enhertu*	Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, PK	• FPCD: Q1 2021
Phase II **DESTINY-CRC01** **NCT03384940**	HER2-expressing advanced colorectal cancer	90	Non randomised single group assignment • *Enhertu*	Primary endpoint ORR Secondary endpoints PFS, OS, DoR, range of PK endpoints	• FPCD: Q1 2018 • LPCD: Q2 2019 • Data readout: Q2 2020 • Primary endpoint met
Phase II **DESTINY-CRC02** **NCT04744831**	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 endpoints PFS, OS, DoR, range of PK endpoints	• FPCD: Q1 2021
Phase I **J101** **NCT02564900**	Advanced solid malignant tumours	278	Non randomised single group assignment • *Enhertu*	Primary end points ORR, number of subjects with AEs, tumour response Secondary endpoints PK	• FPCD: Q3 2015 • Data readout: Q3 2018
Phase I **NCT04042701**	HER2-expressing locally advanced/metastatic breast or NSCLC	115	• Non randomised parallel group assignment • *Enhertu* + pembrolizumab Global trial 2 countries	Primary end points DLT, ORR Secondary endpoints DoR, DCR, PFS, TTR, OS	• FPCD: Q2 2020
Phase I **NCT03523572**	HER2-expressing breast and urothelial cancer	99	• Non randomised sequential assignment • *Enhertu* + nivolumab Global trial 7 countries	Primary end points DLT, ORR, TEAEs Secondary endpoints DoR, DCR, PFS, TTR, OS, ORR (investigator)	• FPCD: Q3 2018

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoint(s)	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 **ACE-CL-311** **NCT03836261**	Previously untreated CLL fit	780	• Arm A; *Calquence* + venetoclax • Arm B: *Calquence* + venetoclax + obinutuzumab • Arm C: FCR or BR	• Primary – IRC PFS (A vs C) • Secondary - IRC PFS (B vs C); INV PFS (A vs C; B vs C)	• FPCD: Q1 2019 • Data anticipated: 2022+
Phase III **ACE-CL-309 (ASCEND)** **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 Q3 2016 • Data readout: Q2 2019 • Primary endpoint met
Phase III **ACE-CL-006 (ELEVATE-RR)** **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: Q2 2015 • Data readout: Q1 2021 • Primary endpoint met
Phase III **ACE-LY-308** **NCT02972840**	Previously untreated MCL	546	• 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: Q1 2017 • Data anticipated: 2022
Phase III **ESCALADE** **NCT04529772**	DLBCL	600	*Calquence* + rituximab, cyclophosphamide, doxorubicin, vincristine, and prednisone	• Safety, ORR	• FPCD: Q2 2020 • Data anticipated: 2022+
Phase II **ACE-CL-208** **NCT02717611**	Relapsed/ refractory CLL, intolerant to ibrutinib	60	*Calquence* monotherapy	• ORR at 36 cycles	• FPCD: Q1 2016 • Data anticipated: H1 2020
Phase II **15-H-0016** **NCT02337829**	Relapsed/refractory and treatment naïve/del17p CLL/SLL	48	*Calquence* monotherapy • Arm A: lymph node biopsy • Arm B: bone marrow biopsy	• ORR	• FPCD: Q4 2014 • Data anticipated: 2022+
Phase I/II **ACE-CL-001** **NCT02029443**	CLL/SLL/Richter's transformation	306	*Calquence* monotherapy Dose escalation and expansion	• Safety, PK, PD	• FPCD: Q1 2014 • Data anticipated: 2021

Calquence (BTK inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase I/II **ACE-LY-001** **NCT02328014**	B-cell malignancies	40	Dose escalation and expansion trial of the combination of *Calquence* and ACP-319 (Pi3K inhibitor)	• Safety • ORR	• FPCD: Q1 2015 • Data anticipated: H1 2020
Phase I/II **ACE-LY-005** **NCT02362035**	Haematological malignancies	161	*Calquence* + pembrolizumab	• Safety • Secondary endpoints: ORR, DoR, PFS, OS, TTNT (time to next therapy)	• FPCD: Q1 2015 • Data anticipated: 2021
Phase I/II **ACE-WM-001** **NCT02180724**	Waldenstrom microglobulinaemia	106	*Calquence* monotherapy	• ORR	• FPCD: Q3 2014 • Data readout: Q4 2019
Phase Ib **ACE-LY-002** **NCT02112526**	Relapsed/refractory de novo activated B-cell DLBCL	21	*Calquence* monotherapy	• Safety	• FPCD: Q3 2014 • Data anticipated: H2 2019
Phase Ib **ACE-LY-106** **NCT02717624**	MCL	70	*Calquence* in combination with bendamustine and rituxumab • Arm A: treatment naive • Arm B: relapsed/refractory • Arm C: treatment naïve: *Calquence* + venetoclax + rituxumab	• Safety	• FPCD: Q1 2016 • Data anticipated: 2022+
Phase Ib **ACE-MY-001** **NCT02211014**	Relapsed/refractory MM	28	• Arm A: *Calquence* • Arm B: *Calquence* + dexamethasone	• Safety	• FPCD: Q1 2015 • Data readout: Q2 2019
Phase I **ACE-LY-003** **NCT02180711**	Relapsed/refractory follicular lymphoma	80	• Arm A: *Calquence* • Arm B: *Calquence* + rituximab • Arm C: *Calquence* + rituximab + lenolidomide	• Safety	• FPCD: Q1 2015 • Data anticipated: 2022+
Phase I **ACE-CL-002** **NCT02157324**	Relapsed/refractory CLL/ SLL	12	*Calquence* in combination with ACP-319 dose escalation	• Safety, PK, PD	• FPCD: Q3 2014 • Data anticipated: H2 2020
Phase I **ACE-CL-003** **NCT02296918**	CLL/SLL/PLL	69	*Calquence* + obinutuzumab • Arm A: relapsed/refractory • Arm B: treatment naïve *Calquence* + venetoclax + rituxumab • Arm C: relapsed/refractory • Arm D: treatment naïve	• Safety, ORR • Secondary endpoints: PD, PFS, TTNT, OS	• FPCD: Q4 2014 • Data anticipated: 2022+

Calquence (BTK inhibitor)

Blood and other cancers

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase I **NCT03198650**	Japanese adults with advanced B-cell malignancies	34	• *Calquence* monotherapy • Dose confirmation and expansion • *Calquence* + obinutuzumab	• Safety • PK	• FPCD: Q2 2017 • Data anticipated: 2022+
Phase I/II **LY-110** **NCT03205046**	B-cell malignancies r/r	25	• Part 1: *Calquence* daily + vistusertib daily • Part 2: *Calquence* daily + vistusertib 5 days on/2 days off	• MTD and optimal dosing schedule • Safety	FPCD: Q3 2017 Data anticipated: H2 2020
Phase III **CL-312** **NCT04008706**	CLL TN and r/r	600	• Arm A: treatment naïve • Arm B: relapsed/refractory • Arm C: prior BTKi therapy • Arm D: concomitant vitamin K antagonists	• Safety	Data anticipated: 2022+
Phase Ib/II **PRISM** **NCT03527147**	Relapsed/refractory aggressive NHL	88	• Arm 1: *Calquence* + danvatirsen • Arm 2: *Calquence* + ceralasertib • Arm 3: *Calquence* + Hu5F9G4 + Rituxan • Arm 4: *Calquence* + AZD5153 An open-label platform trial with trial centres in US and UK	• Primary outcome; safety & tolerability • Secondary outcomes; ORR, DOR, PFS, OS	FPCD: Q2 2018 Data anticipated: 2021
Phase Ib/II **ACE-ST-209** **NCT02586857**	≥ 2L glioblastoma multiforme	52	• Arm A: *Calquence* 200mg BID • Arm B: *Calquence* 400mg QD	• Safety, ORR	• FPCD: Q1 2016 • Data anticipated: H2 2019
Phase I/II **D8220C0007** **NCT03932331**	Chinese adults r/r MCL and r/r CLL	105	• Part 1: R/r B-cell MalignanciesPhase II • Part 2: Cohort A: r/r MCL • Part 2: Cohort B: r/r CLL	• Safety, ORR	• FPCD: Q2 2020
Phase I **D8220C00018** **NCT04488016**	Healthy volunteers	28	Part 1: Rel bioavailabilty for capsule vs tablet Part 2: Rel bioavailabilty for oral solution of tablet	• Safety	• FPCD: Q2 2019 • Data anticipated: H1 2021

Koselugo (selumetinib, MEK inhibitor)

Paediatric neurofibromatosis type 1, solid tumours

Trial	Population	Patients	Design	Endpoints	Status

Phase II SPRINT
 	
NCT01362803
 	
Partnered
	Paediatric NF1	50 (stratum 1)
		25 (Stratum 2)
			• Single arm: *Koselugo* 25mg/m^2 BID with 2 strata:
			  • Stratum 1: PN related morbidity present at enrolment
			  • Stratum 2: no PN related morbidity present at enrolment
				• 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 Ib ***Koselugo* + MK-8353 (ERK inhibitor)** **NCT03745989** **Partnered (Merck Lead trial)**	Advanced solid tumours	80 (dose escalation trial)	Phase Ib open-label trial of MK-8353 in combination with *Koselugo* in participants with advanced solid tumours	• DLTs • AEs • Trial drug discontinuations due to an AE	• FPCD: Q1 2019
Phase I Japan PK / Safety study **Partnered**	Paediatric Inoperable NF1-PN patients	9-12	Open-label Phase I clinical study to assess safety and PK of *Koselugo* in Japanese paediatric NF1-PN patients	• Primary endpoints safety • Secondary endpoints of PK/anti-tumour effect	• FPCD: Q3 2020 • LPCD: Q4 2019
Phase I China PK / Safety / Efficacy study	Pediatric (2-17 years old), adult NF1	32	Single arm with 3 phases; • Dose confirmation phase (n=6 for 3 cycles), • Expansion phase (24mths post LSD) • Long term Follow up (60mths post LSD)	Primary: Safety/tolerability and PK Secondary: Efficacy (ORR, DoR; TTR; PFS)	FPCD: Q4 2020

Lumoxiti (moxetumomab pasudotox,CD22 mAb)

Blood cancer

Trial	Population	Patients	Design	Endpoints	Status

Phase III PLAIT
 	
NCT01829711
 	
Partnered
	Adults with relapsed or refractory HCL	80	• Multicentre, single-arm, open-label Phase III trial
			• *Lumoxiti* i.v. at the recommended dose
				• Primary endpoint: rate of durable CR (complete response): CR maintained for > 180 days
				• Secondary endpoints
				  • Efficacy: CR rate, ORR, Duration of CR and ORR, TTR, PFS
				  • Safety and tolerability
				  • PK and immunogenicity
					• FPCD: Q2 2013
					• Data readout: Q3 2017
					• Primary endpoint met

Savolitinib (MET inhibitor)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT01985555** **Partnered**	Advanced NSCLC (all comers)	85	• Dose escalation trial Conducted in China	• Primary endpoint: safety and tolerability • Secondary endpoint: PK profile	• FPCD: Q2 2013 • Data readout: Q3 2019
Phase II **NCT02897479** **Partnered**	Lung PSC and other NSCLC	65	• Single arm trial: savolitinib QD Conducted in China	• Primary endpoint: ORR • Secondary endpoint: PFS, safety parameters	• FPCD: Q1 2017 • Data readout: Q2 2020
Phase II **NCT04606771**	EGFRm/MET amplified advanced NSCLC	56	• *Tagrisso* and savolitinib contribution of components	• Primary endpoint: ORR • Secondary endpoint: PFS, DoR, OS	• FPCD: Q4 2020 • Data anticipated: 2022

Capivasertib (AKT inhibitor)

Breast cancer, prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPItello-290 **NCT03997123**	Locally advanced or metastatic TNBC	800	Double-blind randomised comparative trial • Arm 1: capivasertib + paclitaxel • Arm 2: placebo + paclitaxel	• OS	• FPCD: Q3 2019 • Data anticipated: 2022+
Phase III CAPItello-291 **NCT04305496**	Locally advanced (Inoperable) or metastatic HR+/HER2− breast cancer	834	Double-blind randomised comparative trial • Arm 1: capivasertib + *Faslodex* • Arm 2: placebo +*Faslodex*	• PFS	• FPCD Q2 2020 • Data anticipated: 2022+
Phase III CAPItello-281 **NCT04493853**	De novo PTEN deficient metastatic hormone sensitive prostate cancer	1,000	Double-blind randomised comparative trial • Arm 1: capivasertib + abiraterone • Arm 2: placebo + abiraterone	• rPFS	• FPCD: Q3 2020 • Data anticipated: 2022+

Monalizumab (NKG2a mAb)

Cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III **INTERLINK-1** **NCT04590963**	Recurrent or Metastatic SCCHN, 2L	600	• Arm A: monalizumab + cetuximab i.v. • Arm B: placebo + cetuximab i.v. Global trial	• Primary: OS • Secondary: PFS, ORR, DoR	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase I/II **NCT02671435**	Advanced solid tumours	381	Escalation phase • monalizumab + *Imfinzi* i.v. Expansion phase • monalizumab + *Imfinzi* i.v. recommended dose Exploration phase • monalizumab + *Imfinzi* i.v. recommended dose + SoC systemic therapy with or without biologic agent and monalizumab in combination with a biologic agent in adult subjects with CRC Global trial	Primary endpoints: • Safety • Exploration Phase: Objective Response per RECIST • Secondary endpoints include tumour response (OR, DC, DoR, PFS and OS), immunogenicity, pharmacokinetics, pharmacodynamics	• FPCD: Q2 2016 • Data anticipated: 2022

Camizestrant (AZD9833, oral SERD)

Breast cancer

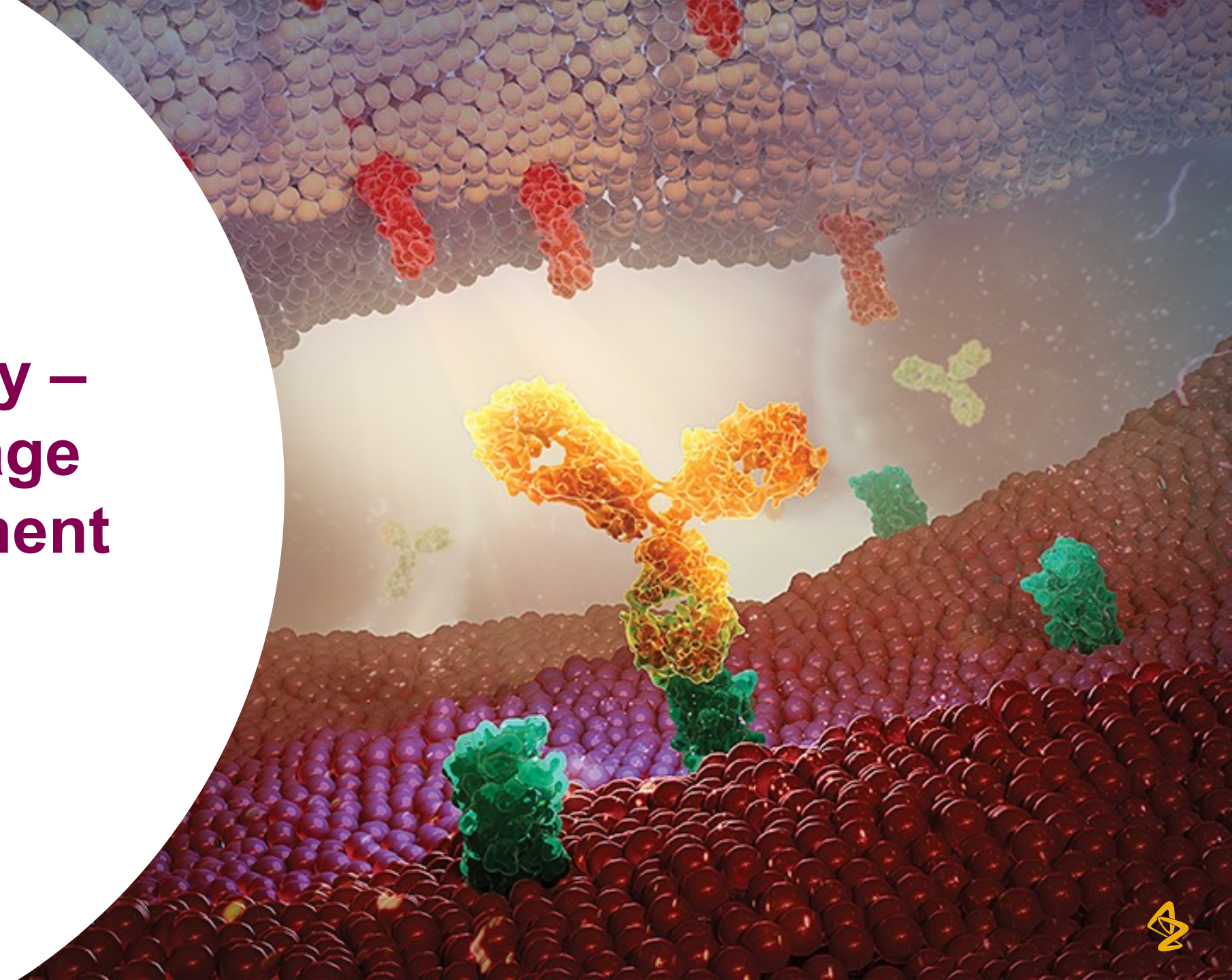
Trial	Population	Patients	Design	Endpoints	Status
Phase III **SERENA-4** **NCT04711252**	ER+ HER2- breast cancer	1,342	A randomised, multicentre, double-blind, Phase III trial of camizestrant plus palbociclib versus anastrozole plus palbociclib for the treatment of patients with oestrogen receptor-positive, HER2-negative advanced breast cancer who have not received any systemic treatment for advanced disease	• Primary endpoint: PFS • Secondary endpoint: OS, PFS2	• FPCD: Q1 2021 • Data anticipated: 2022+
Phase I **NCT03616587**	ER+ breast cancer	266	• Open label multicentre trial of camizestrant administered orally in patients with advanced ER+ HER2 negative breast cancer. The trial design allows an escalation of dose with intensive safety monitoring to ensure the safety of patients. The trial will determine the maximum tolerated dose of AZD9833 as monotherapy and in combination with palbociclib or abemeciclib. In addition, randomised expansion cohort(s) at potential therapeutic dose(s) in patients will be enrolled to further determine the safety, tolerability, pharmacokinetics and biological activity of camizestrant alone and in combination with Palbociclib or abemaciclib	• Primary outcome measures: safety and tolerability • Secondary outcome measures: multiple dose PK of AZD9833 alone and in combination with palbociclib antitumour activity	• FPCD: Q4 2018
Phase II **NCT04214288**	ER+ breast cancer	288	• Randomised, open-label, parallel-group, multicentre trial aimed to compare the efficacy and safety of oral camizestrant versus intramuscular (IM) *Faslodex* in women with advanced breast cancer.	• Primary outcome measure: mPFS	• FPCD: Q2 2020
Phase II **NCT04588298**	ER+ breast cancer	84	• Randomised, open-label, parallel-group, multicentre trial to investigate the biological effects of camizestrant in women with ER positive, HER2 negative primary breast cancer	• Primary outcome measure: change in ER expression between pre- and on-treatment tumour biopsies	• FPCD: Q4 2020
Phase I **NCT04541433**	ER+ breast cancer	18	• Open-label study designed to evaluate the safety, tolerability, pharmacokinetics, and anti-tumour activity of camizestrant in Japanese women with endocrine resistant ER+ HER2- breast cancer that is not amenable to treatment with curative intent.	• Primary outcome measures: safety and tolerability • Secondary outcome measures: multiple dose PK of AZD9833	• FPCD: Q4 2020
Phase I **NCT04546347**	Healthy volunteers	32	• Randomsied, open-label study to determine the relative bioavailability of different oral camizestrant tablet formulations and an camizestrant oral solution, the effect of food on the pharmacokinetics of an oral camizestrant tablet formulation, and the absolute bioavailability of camizestrant study in healthy post-menopausal female volunteers.	• Primary outcome measure: relative bioavailability of AZD9833 delivered as different tablet formulations and the effect of food	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data anticipated: H1 2021

Datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TROPION-LUNG01** **NCT04656652** **Partnered**	NSCLC (without actionable mutation)	590	Randomised, open label • Datopotamab deruxtecan • Docetaxel Global trial	• Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR, TTR, DCR, PK, anti-drug antibodies	• FPCD: Q4 2020 • Data anticipated: 2022+
Phase II **TROPION-LUNG05** **NCT04484142** **Partnered**	NSCLC (with actionable mutation)	150	Randomised, open label • Datopotamab deruxtecan Global trial	• Primary endpoint: ORR • Secondary endpoint: DOR, PFS, OS, safety, PK, anti-drug antibodies	• Initiating
Phase I **NCT03401385** **Partnered**	NSCLC TNBC HR+ BC	350	Open label, two-part (dose escalation, dose expansion) • Datopotamab deruxtecan Japan, US	• Primary endpoint: safety • Secondary endpoint: PK, antitumor activity, anti-drug antibodies	• FPCD: Q1 2018
Phase I **TROPION-LUNG02** **NCT04526691** **Partnered**	NSCLC (without actionable mutation)	86	Open label, combination with pembrolizumab, two-part (dose escalation, dose expansion) • Datopotamab deruxtecan + pembrolizumab Japan, US	• Primary endpoint: safety • Secondary endpoint: ORR, DOR, • PFS, OS, PK, anti-drug antibodies	• FPCD: Q4 2020
Phase I **TROPION-LUNG04** **NCT04612751** **Partnered**	NSCLC (without actionable mutation)	74	Open label, combination with *Imfinzi*, two-part (dose escalation, dose expansion) • Datopotamab deruxtecan + *Imfinzi* US, Japan	• Primary endpoint: safety • Secondary endpoint: : ORR, DOR, • PFS, OS, PK,	• Initiating

Oncology – early-stage development



Imfinzi (PD-L1 mAb)

Cancer

Trial	Compound	Population	Patients	Design	Endpoints	Status
Phase I/II STUDY 1108 NCT01693562	*Imfinzi*	Solid tumours	1,022	• Dose escalation: 5 cohorts at Q2W and 1 cohort at Q3W • Dose expansion: 16 tumour type cohorts at the Q2W MTD defined during dose escalation • Dose exploration: cohort at 20mg Q4W Global trial - nine countries	• Safety • Optimal biologic dose • Secondary endpoints include PK, immunogenicity and antitumour activity	• FPCD: Q3 2012 • LPCD: Q4 2016 • Data readout: Q2 2020
Phase I NCT02117219	*Imfinzi*, azacitidine	Myelodysplastic syndrome	79	Dose escalation and dose expansion trial • Part 1: *Imfinzi* • Part 2 Arm 1: *Imfinzi* and tremelimumab • Part 2 Arm 2: *Imfinzi,* tremelimumab and azacitidine Global trial - four countries	• Safety and tolerability of monotherapy and combination • Secondary endpoints include duration of response, PFS and OS, PK and immunogenicity	• FPCD: Q2 2014 • Data anticipated: Q2 2020
Phase I NCT02900157	MEDI9090	Solid tumours	42	Multi-centre, open-label, single-arm trial for adult subjects US and Japan trial centers	• Safety, PK, number of subjects reporting infusion related reaction	• FPCD: Q3 2016 • LPCD: Q1 2017 • Data readout: Q2 2020
Phase II HUDSON NCT03334617	*Imfinzi* *Lynparza* vistusertib ceralasertib (AZD6738) danvatirsen oleclumab *Enhertu* cediranib	NSCLC	340	5 modules encompassing 16 cohorts • 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 cedirinib • Module 8; Ceralasertib Open-label, biomarker-directed, multi-centre Phase II umbrella trial in patients with NSCLC, who progressed on an anti-PD-1/PD-L1 containing therapy	• Primary outcome; ORR • Secondary outcomes; efficacy including OS, PFS, DCR, and safety and tolerability, DoR	• FPCD: Q1 2018 • Data anticipated: 2022+
Phase II COAST NCT03822351	*Imfinzi*	Stage III NSCLC unresectable	189	• Arm A: *Imfinzi* • Arm B: *Imfinzi* + oleclumab • Arm C: *Imfinzi* + monalizumab	Primary • OR per RECIST v1.1	• FPCD: Q4 2018 • Data anticipated: H2 2021
Phase II NeoCOAST NCT03794544	*Imfinzi*	Resectable, early stage NSCLC	84	• Arm A: *Imfinzi* • Arm B: *Imfinzi* + oleclumab • Arm C: *Imfinzi* + monalizumab • Arm D: *Imfinzi* + danvatirsen	Primary • Major pathological response rate	• FPCD: Q1 2019 • Data anticipated: H2 2021

Imfinzi (PD-L1 mAb)

Cancer

Trial	Compound	Population	Patients	Design	Endpoints	Status
Phase Ib/II COLUMBIA 1 **NCT04068610**	*Imfinzi*	1L metastatic MSS-CRC	112	• Part 1 S1 FOLFOX + bevacizumab + *Imfinzi* + oleclumab • Part 2 Control 1 FOLFOX + bevacizumab • Part 2 E1 FOLFOX + bevacizumab + *Imfinzi* + oleclumab	Primary • Part 1: Safety • Part 2: Efficacy - OR Secondary • Part 1: Efficacy – OR, BOR, DoR, PFS • Part 2: Safety and Efficacy (BOR, DoR, DC, PFS, OS)	• FPCD: Q3 2019 • Data anticipated: H2 2020
Phase I/II SCope-D1	*Imfinzi*	NSCLC SCLC	124	• Open-label, multicentre study to evaluate the safety, pharmacokinetics, and preliminary efficacy of subcutaneous durvalumab in patients with non-small cell and small cell lung cancer	Primary endpoint: • PK, Safety	• FPCD: Q2 2021 • Data anticipated: 2022+

Imfinzi (PD-L1 mAb) + tremelimumab (CTLA-4 mAb)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II **STUDY 22** **NCT02519348**	Hepatocellular carcinoma	545	• Arm A: *Imfinzi* + tremelimumab • Arm B: *Imfinzi* 2L • Arm C: tremelimumab 2L • Arm D: Imfinzi + tremelimumab • Arm E: *Imfinzi* in combination with bevacizumab	• Primary endpoints: Safety & tolerability, DLTs • Secondary endpoints: ORR, DoR, OS	• FPCD: Q4 2015 • Data anticipated: H2 2020
Phase Ib **STUDY 006** **NCT02000947**	NSCLC (Immunotx naïve and Immunotx pretreated patient cohorts)	459	• Dose escalation: minimum 5 cohorts exploring various treme Q4W and *Imfinzi* i.v. Q4W dose combinations, higher dose levels and alternate Q2 schedule added with amendment • Dose expansion: MTD for the combination in escalation to be explored in expansion North American, EU and ROW trial centres	Primary endpoints: • Safety • Optimal biologic dose for the combination • OR • Secondary endpoints include antitumour activity, PK and immunogenicity	• FPCD: Q4 2013 • LPCD: Q4 2016 • Data readout: Q3 2020
Phase I **STUDY 10** **NCT02261220**	Solid tumours (basket trial)	380	• Dose expansion: MTD for the combination in escalation to be explored in expansion cohorts specific for each of 7 tumour types • Dose exploration: 2 cohorts exploring various Q4W treme and *Imfinzi* dose combinations and 2 cohorts exploring various Q2W treme and *Imfinzi* dose combinations North American, EU and ROW trial centres	Primary endpoints: • Safety • Optimal biologic dose for the combination • Secondary endpoints include anti-tumour activity, PK/PD and immunogenicity	• FPCD: Q4 2014 • LPCD: Q2 2017 • Data readout: Q4 2020

Imfinzi (PD-L1 mAb) + MEDI0457 (DNA HPV Vaccine)

Head and neck squamous cell carcinoma (HNSCC)

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/IIa **NCT03162224**	HPV associated recurrent/metastatic head and neck cancer	50	Multi-centre, open label trial to evaluate the safety and efficacy of combination treatment with MEDI0457 and *Imfinzi*	Primary endpoints: Safety & Tolerability, ORR Secondary endpoints: PK, ADA, DCR, OS, PFS	• FPCD: Q3 2017 • Data anticipated: H1 2021

AZD0466 (Bcl2/xL inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04214093**	Advanced hematologic malignancies or solid tumours	10	Monotherapy dose escalation, consisting of two arms: • Arm A: Patients with low risk for tumour lysis syndrome (solid tumours, lymphomas, myelomas) • Arm B: Patients with high risk for tumour lysis syndrome (relapsed/refractory haem malignancies)	• Primary: safety • Secondary: PK, anti-tumour activity	• FPCD: Q4 2019 • Data anticipated: H2 2021

MEDI1191 (IL12 modRNA)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03946800**	Advanced solid tumours	87	First-time-in-human Phase I, open-label, dose-escalation and expansion trial of MEDI1191 administered intratumourally as monotherapy and in combination with *Imfinzi*	• Primary endpoint: safety and tolerability • Secondary endpoints: PK, immunogenicity and efficacy	• FPCD: Q2 2019 • Data anticipated: 2022

AZD1390 (ATM inhibitor)

Cancer

Trial	Population	Subjects	Design	Endpoints	Status
Phase I **NCT03423628**	Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients	132	• Designed to evaluate the safety, tolerability and PK of AZD1390 in combination with radiation therapy in patients with GBM and brain metastases from solid tumours • Dose and schedule of AZD1390 administration will be adjusted during assessment of safety and tolerability during this Phase I trial Conducted across seven sites in USA and UK	• Primary: investigate the safety, tolerability, and MTD of AZD1390 administered in combination with radiation therapy in brain malignancies	• FPCD Q2 2018 • Data anticipated: 2022

Adavosertib (WEE-1 inhibitor)

Ovarian cancer, uterine serous cancer, solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase II **D6010C00004** **NCT02272790**	Platinum-resistant (PR) ovarian cancer	95	• Arm B: paclitaxel + adavosertib • Arm C: carboplatin + adavosertib Global trial	• Primary endpoint: ORR • Secondary endpoints: DoR, PFS, OS, DCR, safety and tolerability	• FPCD: Q1 2015 • LPCD: Q2 2018 • Data readout: Q3 2019
Phase I **D6015C00002** **NCT02617277**	Advanced solid tumours	56	• Dose escalation trial to determine MTD (adavosertib + *Imfinzi*) Conducted in US	• Safety and tolerability	• FPCD: Q4 2015 • LPCD: Q4 2018 • Data readout Q4 2019
Phase II **D601HC00002** **NCT04590248**	Uterine serous carcinoma	120	• Adavosertib monotherapy • Phase IIb, open-label, single-arm, multi-center study • Global trial	• Primary endpoint: ORR • Secondary endpoints: DoR, depth of response, PFS	• FPCD: Q4 2020

AZD2811NP (AURN)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02579226**	Solid tumours	72	• Arm 1: AZD2811NP dose escalation • Arm 2: AZD2811NP dose expansion SCLC	• Safety and tolerability • PK and efficacy	• FPCD: Q4 2015 • Data anticipated: H1 2021
Phase I **NCT03217838**	AML/high-risk MDS	124	• Part A: AZD2811NP monotherapy / azacitidine combination / venetoclax combination dose escalation cohorts • Part B: AZD2811NP monotherapy / azacitidine combination / venetoclax combination dose expansions to further explore the tolerability, PK and clinical activity	• Safety and tolerability • PK and efficacy	• FPCD: Q3 2017 • Data anticipated: 2022+

AZD4573 (CDK9 inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03263637**	Relapsed/refractory haematologic malignancies	45	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 Trial conducted in NL, UK, GE	Primary: • safety/PK; Secondary: • efficacy	• FPCD: Q4 2017 • Data anticipated: H2 2021
Phase I/II **NCT04630756**	Relapsed/refractory haematologic malignancies	78	Modular design platform trial: • Module 1: AZD4573 + *Calquence* (100mg twice daily) combination • Arm 1: dose setting (DLBCL, all comers); ramp-up across 3 dose levels (Part A) • Arm 2: dose expansion (GCB vs. non-GCB DLBCL); target dose (Part B) • I.V. route of administration • Trial conducted in10 countries across North America, EU, ROW	Primary: • Safety (Part A) • ORR (Part B) Secondary: • Safety, anti-tumour activity (Part B) • PK (Parts A & B)	• FPCD: Q1 2021

Imaradenant (AZD4635, $A_{2A}R$ inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02740985**	Phase Ia: patients with advanced solid tumours Phase Ib: Post-immunotherapy NSCLC Other post-immunotherapy solid tumours Immune checkpoint-naïve mCRPC Immune checkpoint-naïve CRC Other immune checkpoint-naïve solid tumours	313	Phase Ia – solid tumours or mCPRC: • Imaradenant monotherapy • Imaradenant + *Imfinzi* • Imaradenant + abiraterone • Imaradenant + enzalutamide • Imaradenant + *Imfinzi* + oleclumab • AZD4635 + docetaxel. Phase Ib: Imaradenant monotherapy or Imaradenant + *Imfinzi* dose expansions in NSCLC, mCRPC, CRC and other post-immunotherapy and immune checkpoint-naïve solid tumours Conducted at sites in the US	Primary outcome measure: • Safety and tolerability Secondary outcome measures: • Preliminary assessment of anti-tumour activity	• FPCD: Q2 2016 • Data anticipated: H2 2021
Phase I **NCT03710434**	Healthy male volunteers	21	• Part A 2-period randomised crossover trial of single doses of Imaradenant, nanosuspension or solid oral formulation in fasted state • Part B, 4-period, open-label, randomised, crossover trial of single doses of Imaradenant in the same subjects from Part A to assess food effect, pH effect and formulation variants Both parts conducted at a site in the UK	Primary outcome measures: • Cmax and exposure (AUC) of Imaradenant solid oral formulation and nano-suspension	• FPCD: Q4 2018 • LPCD: Q2 2019
Phase II **NCT04089553**	Prostate cancer	60	ARM 1: Imaradenant + *Imfinzi* ARM 2: Imaradenant + oleclumab Conducted at sites in the US	• Primary outcome measure: Efficacy; (ORR and PSA response) • Secondary outcome measure: Efficacy, PK, safety and tolerability	• FPCD: Q3 2019 • Data anticipated: H2 2021
Phase I **NCT03980821**	Japanese patients with advanced solid malignancies	12	Imaradenant dose escalation Conducted at sites in Japan	Primary outcome measure: • Safety and tolerability Secondary outcome measure: • PK and preliminary anti-tumour activity	• FPCD: Q3 2019 • LPCD: Q3 2020
Phase II **NCT04495179**	Prostate cancer	80	ARM A: Imaradenant + *Imfinzi* ARM B: Imaradenant+ *Imfinzi* + cabazitaxel Conducted at sites in US, Europe, UK and Korea	• Primary outcome measure: Efficacy (rPFS) • Secondary outcome measure: Efficacy (OS, PSA response, ORR, DoR)	• FPCD: Q3 2020 • Data anticipated: 2022+

AZD5305 (PARP inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04644068**	Advanced, metastatic HER2 neg. with BRCAm, PALB2m or RAD51C/Dm Breast cancer Advanced, metastatic TNBC BRCAm, PALB2m or RAD51C/Dm PSR ovarian cancer HRD+ve1 (non-BRCAm or PALB2m or RAD51C/Dm) PSR ovarian cancer PSR ovarian cancer	612	A modular phase I/IIa, open-label, multicentre trial to assess the safety, tolerability, pharmacokinetics, pharmacodynamics and preliminary efficacy of ascending doses of AZD5305 as monotherapy and in combination with anti-cancer agents in patients with advanced solid malignancies	• Primary endpoint: safety/tolerability & PK • Secondary endpoints: efficacy	• FPCD: Q4 2020 • Data anticipated: 2022+

MEDI5395 (rNDV GMCSF)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03889275**	Select advanced solid tumours	188	First-time-in-human Phase I, open-label, dose-escalation and expansion arm of MEDI5395 in combination with *Imfinzi*	• Primary endpoint: safety and tolerability • Secondary endpoints: PK, PD, immunogenicity and efficacy	• FPCD: Q4 2019 • Data anticipated: 2022+

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

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa **NCT03530397**	Advanced solid tumours	261	Open-label, dose-escalation and dose-expansion: • Dose-escalation: MEDI5752 i.v. • Dose-expansion: MEDI5752 i.v. as monotherapy and in combination with chemotherapy • Arm A: MEDI5752 i.v. • Arm B: MEDI5752 i.v., pemetrexed and carboplatin • Arm C: Pembrolizumab, pemetrexed and carboplatin	Primary endpoints: • dose-escalation: safety & determination of MTD • dose-expansion: assessment of antitumour activity based on OR Secondary endpoints: • PK, ADA, tumoural baseline PD-L1, assessment of antitumour activity based on OR, DoR, DCR, PFS, OS	• FPCD: Q2 2018 • Data anticipated: 2022+
Phase Ib **NCT04522323**	Advanced renal cell carcinoma	77	Open-label, dose-escalation and dose-expansion to explore the safety, tolerability and anti-tumour activity of MEDI5752 in combination with axitinib:	Primary endpoint: • dose-escalation: safety & tolerability Secondary endpoints: • PK, ADA and antitumour activity of MEDI5752 + axitinib based on PFS, OR, DoR, DCR, TTR, OS	• FPCD: Q3 2020 • Data anticipated: 2022+

AZD5991 (MCL1 inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib/IIa **NCT03218683**	Relapsed/refractory haematologic malignancies	121	• Arm1: monotherapy dose escalation & expansions in relapsed/refractory haematological malignancies • Arm2: combination dose escalation (AZD5991+venetoclax) in relapsed/refractory AML/MDS;. • i.v. route of administration • US only	• Primary: safety • Secondary: PK, efficacy	• FPCD: Q3 2017 • Data anticipated: H2 2021

Ceralasertib (AZD6738, ATR inhibitor)

Cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02264678**	Solid tumours	250	• Arm 1: ceralasertib + carboplatin • Arm 2: ceralasertib dose escalation, ceralasertib + *Lynparza* • Arm 3: ceralasertib + *Imfinzi* Trial conducted in North America, Europe and South Korea	• Safety and tolerability • PK and efficacy	• FPCD: Q4 2014 • Data anticipated: 2022+
Phase I **NCT03022409**	HNSCC	44	Window of opportunity • Arm 1: ceralasertib • Arm 2: *Lynparza* Trial conducted in US, France, Taiwan and the UK	• Biomarker change	• FPCD: Q4 2017 • Data anticipated: H2 2021
Phase II **PLANETTE** **NCT04564027**	Solid tumours mCRPC	52	• Cohort A: ceralasertib; ATM-altered AST • Cohort B: ceralasertib; ATM-altered mCRPC	Cohort A: ORR Cohort B: Composite RR	• FPCD: Q1 2021 • Data anticipated: 2022+

AZD7648 (selective DNA-PK inhibitor)

Advanced solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03907969**	Advanced malignancies	234	• First in human modular dose escalation and dose expansion trial • Arm 1 - AZD7648 monotherapy • Arm 2 - AZD7648 + Pegylated Liposomal Doxorubicin • Arm 3 - AZD7648 + *Lynparza* • Countries: US, UK	• Primary outcome measures: safety and tolerability • Secondary outcome measures: PK, Cytochromes P450, preliminary anti-tumour activity	• FPCD: Q4 2019 • Data anticipated: 2022

AZD8701 (FOXP3 antisense oligonucleotide)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib NCT04504669	Advanced solid tumours	123	Dose escalation and dose expansion trial Arm 1: AZD8701 monotherapy Arm 2: AZD8701 & *Imfinzi* combination therapy Global trial - four countries - US, CA, FR, ES i.v. route of administration	Primary endpoints: safety & tolerability Secondary endpoints: PK, PD, preliminary anti-tumour activity	• FPCD: Q3 2020 • Data Anticipated: 2022+

MEDI9253 (rNDV-IL12)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04613492**	Advanced solid tumours	86	First-time-in-human Phase I, open-label, dose-escalation and expansion arm of MEDI9253 in combination with *Imfinzi*	• Primary endpoint: safety and tolerability • Secondary endpoints: PK, PD, immunogenicity and efficacy	• FPCD: Q4 2020 • Data anticipated: 2022+

Oleclumab (CD73 mAb)

Cancer

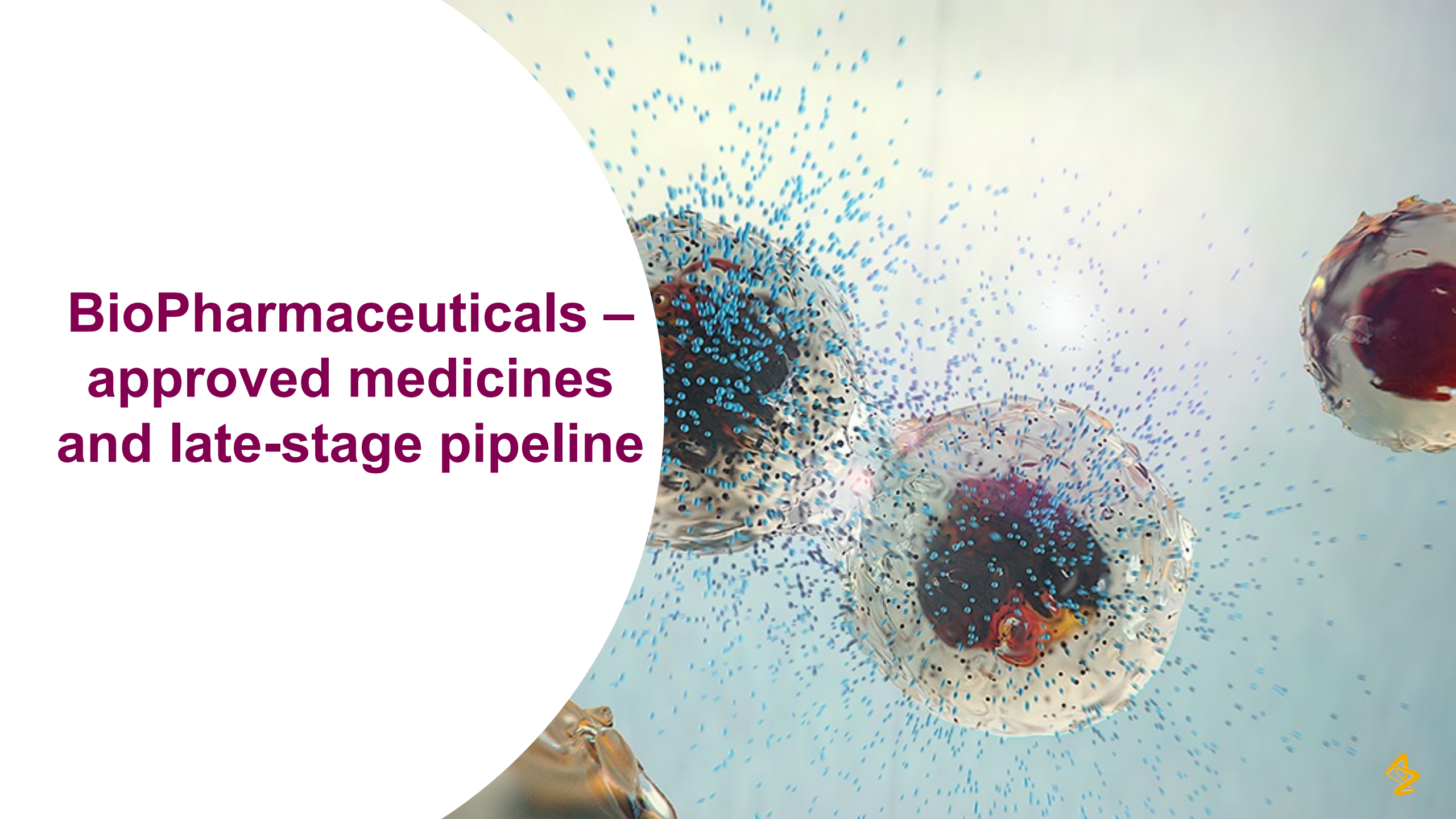
Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02503774**	Advanced malignancies	348	Dose escalation phase • oleclumab i.v. • oleclumab i.v. + *Imfinzi* i.v. Dose expansion phase • oleclumab i.v. recommended dose + *Imfinzi* i.v. US, South Korean and Australian trial centres	Primary endpoints: • Safety • Determination of MTD • Secondary endpoints include preliminary anti-tumour activity, PK, PD, immunogenicity and biomarker activity	• FPCD: Q3 2015 • Data anticipated: H1 2021
Phase Ib/II **NCT03611556**	Pancreatic 1L and 2L with prior gemcitabine-based chemotherapy	339	• Arm A1: gemcitabine and nab paclitaxel i.v. • Arm A2: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. • Arm A3: gemcitabine and nab paclitaxel i.v. + oleclumab i.v. + Imfinzi i.v. • Arm B1: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. • Arm B2: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. • Arm B3: mFOLFOX (oxaliplatin, leucovorin, 5-FU) i.v. + oleclumab i.v. + *Imfinzi* i.v. US, Norway, Spain and 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: H2 2021

IPH5201 (CD39 mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04261075** **Partnered**	Advanced Solid tumours	204	• First time in human Phase I, 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: IV • Geographical Regions: 4 countries - US and 3 in EU.	Primary endpoints: AE, SAE, DLT Secondary endpoints: OR, DC, PK, ADA	• FPCD: Q1 2020 • Data anticipated: 2022

BioPharmaceuticals – approved medicines and late-stage pipeline



Farxiga (SGLT2 inhibitor)

Heart failure and chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III **Dapa-CKD** **NCT03036150**	Patients With CKD	4,304	• Arm 1: *Farxiga* 10mg or 5 mg QD • Arm 2: placebo Global trial - 21 countries	• Primary endpoint: time to the first occurrence of any of the components of the composite: ≥50% sustained decline in eGFR or reaching ESRD or CV death or renal death	• FPCD: Q1 2017 • LPCD: Q1 2020 • Data readout: Q2 2020 • Primary endpoint met
Phase III **DELIVER** **NCT03619213**	CHF patients with HFpEF	6,100	• 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: Q4 2020 • Data anticipated: H2 2021
Phase III **DAPA-MI** **NCT04564742**	Patients with myocardial infarction	6,400	• 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: hospitalization for HF or CV death	• FPCD: Q4 2020 • Data anticipated: 2022+

Brilinta (P2Y12 receptor antagonist)

Cardiovascular risk reduction

Trial	Population	Patients	Design	Endpoints (primary)	Status
Phase III **THALES** **NCT03354429**	Patients with acute ischaemic stroke or transient ischaemic attack	11,000	• Arm 1: *Brilinta* 90mg BiD • Arm 2: placebo BiD on a background of acetylsalicylic acid if not contra-indicated or not tolerated Global trial – 28 countries	Primary endpoint: • Prevention of the composite of subsequent stroke and death at 30 days Secondary endpoints include: • Prevention of subsequent ischaemic stroke at 30 days • Reduction of overall disability at 30 days	• FPCD: Q1 2018 • LPCD: Q4 2019 • Data readout: Q1 2020 • Primary endpoint met

Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II **PRIORITIZE HF** **NCT03532009**	Patients with chronic heart failure and hyperkalaemia or at high risk of developing hyperkalaemia	182	• Arm 1: *Lokelma* 5g QD for 12 weeks. Option to uptitrate to 10 and 15g QD or downtitrate to 5g QOD • Arm 2: placebo QD for 12 weeks Global trial – nine countries	• Primary endpoint: difference between *Lokelma* and placebo in RAAS (renin–angiotensin–aldosterone system) blockade treatment.	• FPCD: Q3 2018 • LPCD: Q2 2020 • Data readout: Q4 2020
Phase IIIb **DIALIZE China** **NCT04217590**	Patients with ESRD with hyperkalemia and on stable haemodialysis	134	• Arm 1: Lokelma 5g QD for 8 weeks on non-dialysis days. Option to uptitrate to 10 and15g 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 • Data readout: H2 2021
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, India	• Primary endpoint: maintenance of normokalaemia	• Initiating • Data readout: 2022+
Phase III **DIALIZE-Outcomes** **NCT04847232**	Patients with with recurrent hyperkalemia on chronic haemodialysis	2,300	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 hospitalization/intervention/ED visit due to arrhythmias	• Initiating • Data readout: 2022+

Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III **ANDES** **NCT01750190** **Partnered**	Anaemia in CKD in patients not receiving dialysis	922	• Arm 1: roxadustat • Arm 2: placebo Global trial	• Primary endpoint: Haemoglobin response	• FPCD: Q4 2012 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by FibroGen
Phase III **ALPS** **NCT01887600** **Partnered**		597	• Arm 1: roxadustat • Arm 2: placebo Global trial	• Primary endpoint: Haemoglobin response	• FPCD: Q2 2013 • LPCD: Q4 2017 • Data readout: Q3 2018 • Primary endpoint met Sponsored by Astellas
Phase III **DOLOMITES** **NCT02021318** **Partnered**		616	• Arm 1: roxadustat • Arm 2: darbepoetin alfa Global trial	• Primary endpoint: Haemoglobin response	• FPCD: Q1 2014 • LPCD: Q4 2019 • Data readout: Q1 2020 • Primary endpoint met Sponsored by Astellas
Phase III **OLYMPUS** **NCT02174627**		2,781	• Arm 1: roxadustat • Arm 2: placebo Global trial	• Primary efficacy endpoint: Haemoglobin response • Primary safety objective: Contribute CV safety data to pooled safety • analyses across the Phase III program	• FPCD: Q3 2014 • LPCD: Q4 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by AstraZeneca
Phase III **ROCKIES** **NCT02174731**	Anaemia in CKD in patients receiving dialysis	2,133	• Arm 1: roxadustat • Arm 2: epoetin alfa Global trial	• Primary efficacy endpoint: Haemoglobin response • Primary safety objective:Contribute CV safety data to pooled safety • analyses across the Phase III program	• FPCD: Q3 2014 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by AstraZeneca
Phase III **SIERRAS** **NCT02273726** **Partnered**		741	• Arm 1: roxadustat • Arm 2: epoetin alfa Global trial	• Primary endpoint: Haemoglobin response	• FPCD: Q4 2014 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by FibroGen
Phase III **PYRENEES** **NCT02278341** **Partnered**		838	• Arm 1: roxadustat • Arm 2: epoetin alfa or darbepoetin alfa Global trial	• Primary endpoint: Haemoglobin response	• FPCD: Q4 2014 • LPCD: Q3 2018 • Data readout: Q3 2018 • Primary endpoint met Sponsored by Astellas

Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III **HIMALAYAS** **NCT02052310** **Partnered**	Anaemia in newly initiated dialysis patients	1,043	• Arm 1: roxadustat • Arm 2: epoetin alfa Global trial	• Primary endpoint: Haemoglobin response	• FPCD: Q4 2013 • LPCD: Q3 2018 • Data readout: Q4 2018 • Primary endpoint met Sponsored by FibroGen
Phase III **NCT03263091** **Partnered**	Anaemia in lower risk MDS patients	184	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: 2022 Sponsored by FibroGen
Phase II/III **NCT03303066** **Partnered**	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: 2022 Sponsored by FibroGen
Phase II **NCT04076943** **Partnered**	Anemia in patients receiving chemotherapy treatment for non-myeloid malignancies	100	US	• Primary endpoint: Maximum change in hemoglobin within 16 weeks from baseline without RBC transfusion	• FPCD: Q3 2019 • LPCD: Q3 2020 • Data anticipated: H1 2021 Sponsored by FibroGen

Eklira/ Tudorza (LAMA, DPI)

COPD

Trial	Population	Number of patients	Design	Endpoints	Status
Phase I **NCT03276052**	Healthy Chinese volunteers	20	Open-label, 2-period ascending dose incomplete block, cross-over trial • aclidinium bromide 400 µg DPI Global trial – one Country	• To investigate the PK of aclidinium bromide and its metabolites after single and multiple doses (BID) of aclidinium bromide 200 µg, 400 µg and 800 µg • To evaluate the safety, and tolerability of aclidinium bromide 200 µg, 400 µg and 800 µg after single and multiple dose administration (BID)	• Initiating • Data anticipated: 2022+

Duaklir Genuair (LAMA/LABA, DPI)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **AVANT** **NCT03022097**	Patients with stable COPD	1,060	• Arm 1: *Duaklir Genuair* 400/12 µg DPI • Arm 2: aclidinium bromide 400 µg DPI • Arm 3: formoterol fumarate 12 µg DPI • Arm 4: tiotropium 18 µg DPI Global trial – five countries	Primary endpoints: • Change from baseline in one hour morning post-dose dose FEV1 *Duaklir Genuair* 400/12 µg compared to Aclidinium bromide at Week 24 • Change from baseline in morning pre-dose (trough) FEV1 of *Duaklir Genuair* 400/12 µg compared to Formoterol fumarate at Week 24 • Change from baseline in trough FEV1 of Aclidinium bromide 400 µg compared to placebo at Week 24	• FPCD: Q1 2017 • Data anticipated: 2022+

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

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III KALOS **NCT04609878**	Severe asthma	2,800	Treatments (24 to 52 week variable length) • BGF MDI 320/28.8/9.6µg BID pMDI • BGF MDI 320/14.4/9.6µg BID pMDI • BFF MDI 320/9.6µg BID pMDI • *Symbicort* 320/9µg BID pMDI Randomised, double-blind, double dummy, parallel group and multicentre 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 Studies 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: 2022+
Phase III LOGOS **NCT04609904**	Severe asthma	2,800	Treatments (24 to 52 week variable length) • BGF MDI 320/28.8/9.6µg BID pMDI • BGF MDI 320/14.4/9.6µg BID pMDI • BFF MDI 320/9.6µg BID pMDI • *Symbicort* 320/9µg BID pMDI Randomised, double-blind, double dummy, parallel group and multicentre 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 Studies 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: 2022+

Daliresp/Daxas (PDE4 inhibitor, oral)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Post Launch PASS **NCT03381573**	COPD	124,080	• This is 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), and Sweden (national databases including healthcare, death, and demographics data).	• Primary endpoint: all-cause mortality (up to five years)	• Data anticipated: 2022+

Fasenra (IL5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELTEMI **NCT02808819**	A multi-centre, open-label, safety extension trial with *Fasenra* for asthmatic adults on ICS plus LABA2 Agonist Age 18-75 years	447	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: *Fasenra* 30mg Q8W s.c. Global trial - 15 countries	• Primary endpoint: safety and tolerability	• FPCD: Q2 2016 • LPCD: Q3 2019 • Data readout: Q3 2020 • Primary endpoint met
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
D3250C00036 China ICS/LABA Trial (MIRACLE) **NCT03186209**	Severe, uncontrolled asthma, despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 12-75 years	666	• 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 • Data readout: 2022+

Fasenra (IL5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **BORA** **NCT02258542**	Severe asthma, inadequately controlled despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 12-75 years	2,133	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: *Fasenra* 30mg Q8W s.c.* • placebo administered at select interim visits to maintain blind between treatment arms 56-week (adults) 108-week (adolescents) Global trial – 24 countries	• Primary endpoint: safety and tolerability	• FPCD: Q4 2014 • Data readout: Q3 2018 • Primary endpoint met
Phase III **GREGALE** **NCT02417961**	Severe asthma, inadequately controlled despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 18-75 years	162	• Arm 1: *Fasenra* 30mg Q4W s.c. 28-week (adults) Global trial – two countries	• Primary endpoint: functionality, reliability, and performance of a pre-filled syringe with *Fasenra* administered at home	• FPCD: Q2 2015 • Data readout: Q2 2016 • Primary endpoint met
Phase III **ARIA** **NCT02821416**	A double-blind, randomised, parallel group, placebo-controlled multi-centre trial to evaluate the effect of *Fasenra* on allergen-induced inflammation in Mild, atopic asthmatic Age 18-65 years	46	• Arm 1 : *Fasenra* 30mg Q4W s.c. • Arm 2: placebo s.c. 37-week trial	• Primary endpoint: safety and tolerability • Primary endpoint: the effect of *Fasenra* on allergen induced eosinophil changes in sputum and allergen-induced late asthmatic response	• FPCD Q4 2016 • LPCD: Q2 2019 • Data readout: Q4 2020 • Primary endpoint met
Phase III **ALIZE** **NCT02814643**	A multi-centre, randomised, double-blind, parallel group, placebo-controlled, Phase IIIb trial to evaluate the potential effect of *Fasenra* on the humoral immune response to the seasonal influenza vaccination in adolescent and young adult patients with severe asthma Ages 12-21 years	103	• Arm 1: *Fasenra* 30mg Q4W s.c. with one dose of seasonal influenza virus vaccine IM • Arm 2: placebo Q4W s.c. with one dose of seasonal influenza virus vaccine intra muscular 12-week trial	Primary endpoints: • Post-dose strain-specific HAI) antibody GMFRs • Post-dose strain-specific serum HAI antibody GMTs • Proportion of patients who experience a strain-specific post-dose antibody response with antibody response defined as a ≥4-fold rise in HAI antibody titer	• FPCD: Q3 2016 • Data readout: Q3 2017 • Primary endpoint met

Fasenra (IL5R mAb)

Severe, uncontrolled asthma, COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase III **GRECO** **NCT02918071**	Severe asthma on ICS-LABA Age 18-75 years	121	Open label *Fasenra* 30mg Q4w 28-week trial Global trial - two countries	• Primary endpoint: percentage of patients/ caregivers who successfully self administer at home	• FPCD: Q4 2016 • Data readout: Q4 2017 • Primary endpoint met
Phase IIIb **ANDHI** **NCT03170271**	A multi-centre, randomised, double-blind, parallel group, placebo controlled, Phase IIIb trial to evaluate the safety and efficacy of *Fasenra* 30 mg s.c. in patients with severe asthma uncontrolled on SoC treatment. Age 18-75	659	• Arm 1: *Fasenra* 30mg Q8W s.c. • Arm 2: placebo s.c. 24-week trial Global trial – 15 countries	• Primary endpoint: rate of asthma exacerbations • Secondary outcome measures: Saint George Respiratory Questionnaire (SGRQ)	• FPCD: Q3 2017 • LPCD: Q1 2019 • Data readout: Q4 2019 • Primary endpoint met
Phase I **AMES** **NCT02968914**	Healthy volunteers age 18-55 years	180	Open label trial to compare 30 mg *Fasenra* PK administered by APFS or AI device 8-week trial Global trial – two countries	• Primary endpoint: PK comparability	• FPCD: Q1 2017 • Data readout: Q3 2017
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	868	• Double-blind, placebo controlled, single dose (100mg q8w) • 56-week treatment • Global trial – 26 countries	• Primary endpoint: annualized rate of moderate or severe exacerbations over 56 weeks	• FPCD Q4 2019 • Data anticipated: 2022+

Fasenra (IL5R mAb)

Nasal polyposis and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III OSTRO **NCT03401229**	Patients with severe bilateral nasal polyposis 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 enpoints met
Phase III ORCHID **NCT04157335**	Patients with eosinophilic chronic rhinosinusitis with severe nasal polyposis Age 18-75 years	148	Arm 1: *Fasenra* 30mg Q8W s.c. Arm 2: placebo Q8W s.c. 56-week trial Asian countries (4 countries)	• Primary endpoint: Change in endoscopic total nasal polyp score and Change in mean nasal blockage score	• FPCD: Q4 2019 • Data anticipated: 2022+
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: 2022+
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: 2022
Phase III MESSINA **NCT04543409**	Documented diagnosis of EoE Age 12 to 65 years	170	• Arm 1: *Fasenra* 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. 24-week double blind treatment period and open label period(s) Global trials – 12 countries	• Primary endpoints: Histologic response at week 24 Change from baseline in DSQ score at week 24	• FPCD Q4 2020 • Data anticipated: 2022

Fasenra (IL5R mAb)

Dermatology

Trial	Population	Patients	Design	Endpoints	Status
Phase III **FJORD** **NCT04612790**	Patients with symptomatic (newly diagnosed or relapsing) Bullous Pemphigoid	120	• Arm 1: *Fasenra* regimen • Arm 2: placebo 36-week double blind treatment period and open label period Global trial	• Primary endpoint: Proportion of patients with complete sustained (≥2 months) remission off OCS at 36 weeks	• FPCD: Q2 2021 • Data anticipated: 2022+
Phase II **ARROYO** **NCT04612725**	Patients with moderate/severe Chronic Spontaneous Urticaria, and resistant to H1 treatment	160	• Arm 1: *Fasenra* regimen 1 • Arm 2: *Fasenra* regimen 2 • Arm 3: placebo 24-week double blind treatment period and open label period Global trial	• Primary endpoint: Change from baseline in ISS7 at week 12	• FPCD: Q4 2020 • Data anticipated: 2022
Phase II **HILLIER** **NCT04605094**	Patients with moderate to severe Atopic Dermatitis despite treatment with topical medications	160-200	• Arm 1: *Fasenra* regimen • Arm 2: placebo 16-week double blind treatment period and open label periods Global trial	• Primary endpoint: Proportion of patients with an IGA 0/1 and a decrease in IGA of ≥ 2 points at week 16	• FPCD: Q4 2020 • Data anticipated: 2022+

Tezepelumab (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR **NCT03347279** **Partnered**	Severe asthma Age 12-80 years	1,061	• Arm 1: tezepelumab 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**	Severe asthma Age 18-80 years	150	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 48 week trial Global trial – seven 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**	Severe asthma Age 12-80 years	~975	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. Extension trial to NAVIGATOR and SOURCE. 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 anticipated: H2 2022
Phase III PATH-HOME **NCT03968978** **Partnered**	Severe asthma Age 12-80 years	216	• Arm 1: tezepelumab s.c. via autoinjector (AI) • Arm 2: tezepelumab s.c. via accessorized pre-filled syringe (APFS) 24 week trial Global trial – 4 countries	Primary endpoint: Proportion of health care professionals and patients /caregivers who successfully administrated tezepelumab 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

Tezepelumab (TSLP mAb)

Severe, uncontrolled asthma, COPD & CRSwNP

Trial	Population	Patients	Design	Endpoints	Status
Phase II CASCADE **NCT03688074** **Partnered**	Severe asthma Age 18-75 years	116	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 28 week trial Global trial – five countries	• Primary endpoint: number of airway submucosal inflammatory cells/mm2 of bronchoscopic biopsies	• FPCD: Q4 2018 • LPCD: Q4 2019 • Data anticipated: H1 2021
Phase III DIRECTION **NCT03927157** **Partnered**	Severe asthma Age 18-80 years	396	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 52 week trial Regional Asia trial – three 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
Phase III NOZOMI **NCT04048343** **Partnered**	Severe asthma 12-80 years	65	Arm 1: tezepelumab s.c. 52 week trial Local trial - Japan	• Primary endpoint: Number of patients with adverse events	• FPCD: Q2 2019 • LPCD: Q4 2019 • Data anticipated: H1 2021
Phase IIa COURSE **NCT04039113** **Partnered**	Moderate to very severe COPD Age 40-80	282	• Arm 1: tezepelumab 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: 2022+
Phase III WAYPOINT **NCT04851964** **Partnered**	Severe Chronic Rhinosinusitis with Nasal Polyps (CRSwNP) Age 18+	400	• Arm 1: tezepelumab s.c. • Arm 2: placebo s.c. 52 week trial Global trial – 11 countries	• Co-primary endpoint: Nasal Polyp Score and Participant Reported Nasal Congestion	• Initiating

PT027 (SABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III **MANDALA** **NCT03769090** **Managed by Avillion**	Moderate to severe asthma	3,100	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 Randomised, double-blind, multi-centre, parallel group 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 • Data anticipated: H2 2021
Phase III **DENALI** **NCT03847896** **Managed by Avillion**	Mild to moderate asthma	1,000	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 Randomised, double-blind, multi-centre and parallel-group 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 anticipated: H2 2021
Phase III **TYREE** **NCT04234464** **Managed by Avillion**	Asthma with exercise induced bronchoconstriction	60	Treatments (single dose) • BDA MDI 160/180 µg • placebo MDI QID Randomised, double-blind, multi-centre crossover Country: 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

Anifrolumab (type I interferon receptor mAb)

Lupus (SLE / LN)

Trial	Population	Patients	Design	Endpoints	Status
Phase III **TULIP SLE 1** **NCT02446912**	Moderate to severe SLE	450	• Arm 1: 300mg i.v. anifrolumab Q4W for 48 weeks • Arm 2: 150mg i.v. anifrolumab Q4W for 48 weeks • Arm 3: placebo i.v. Q4W for 48 weeks	• Primary endpoint: response in SLE responder index at week 52	• FPCD: Q4 2015 • LPCD: Q4 2017 • Data readout: Q3 2018 • Primary endpoint not met
Phase III **TULIP SLE 2** **NCT02446899**	Moderate to severe SLE	360	• Arm 1: 300mg i.v. anifrolumab Q4W for 48 weeks • Arm 2: placebo i.v. Q4W for 48 weeks	• Primary endpoint: response in SLE responder index at week 52 BICLA at week 52	• FPCD: Q4 2015 • LPCD: Q4 2017 • Data readout: Q3 2019 • Primary endpoint met
Phase III **TULIP LTE** **NCT02794285**	Moderate to severe SLE	630	• Arm 1: 300mg i.v. anifrolumab 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: 2022
Phase II **NCT01438489**	Moderate to severe SLE patients	307	• Arm 1: 300mg i.v. anifrolumab Q4W for 48 weeks • Arm 2: 1000mg i.v. anifrolumab Q4W for 48 weeks • Arm 3: placebo i.v. Q4W for 48 weeks	• Primary endpoint: response in SLE responder index at 6 months	• FPCD: Q1 2012 • LPCD: Q1 2015 • Data readout: Q3 2014
Phase II **NCT01753193**	Moderate to severe SLE patients	218	• Arm 1: anifrolumab, i.v. Q4W for 104 weeks	• Primary endpoint: open-label extension to evaluate long-term safety and tolerability	• FPCD: Q1 2013 • Data readout: Q4 2018
Phase II **NCT02962960**	Moderate to severe SLE patients	32	• Arm 1: 150mg s.c. every other week • Arm 2: 300mg s.c. every other week • Arm 3: placebo s.c. every other week	• PK/PD, safety, tolerability, primary analysis at week 12, secondary analysis at week 52	• FPCD: Q1 2017 • LPCD: Q4 2017 • Data readout: Q1 2018
Phase II **TULIP-LN1** **NCT02547922**	Active Proliferative LN	150	• Arm 1: 900 mg i.v. Q4W for 12 weeks then 300mg i.v. anifrolumab Q4W for 36 weeks • Arm 2: 300 mg i.v. anifrolumab Q4W for 48 weeks • Arm 3: placebo i.v. Q4W for 48 weeks	• Response in proteinuria at week 52	• FPCD: Q4 2015 • LPCD: Q4 2018 • Data readout: Q1 2021

Brazikumab (IL23 inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb / III **INTREPID** **NCT03759288**	Crohn's Disease	1,140	• Arm 1: brazikumab high IV dose on day 1, 29 and 57 + SC brazikumab on day 85 and every 4 weeks through week 48 • Arm 2: brazikumab low IV dose on day 1, 29 and 57 SC brazikumab on day 85 and every 4 weeks through week 48 • Arm 3: adalimumab SC on day 1, 15, 29 and every 2 weeks through week 50 • Arm 4: placebo	Primary • Endoscopic response and clinical remission at week 12 Secondary • Endoscope response and clinical remission at both weeks 12 and 52 • Endoscopic remission and clinical remission at week 52	• FPCD: Q4 2018 • Data anticipated: 2022+
Phase III **NCT03961815**	Crohn's Disease	1,000	• Open label extension	• Safety of long-term treatment with brazikumab	• FPCD: Q2 2019 • Data anticipated: 2022+
Phase II **EXPEDITION** **NCT03616821**	Ulcerative Colitis	375	• Arm 1: brazikumab dose 1 IV on day 1, 15 and 43 + SC brazikumab from day 71 and every 4 weeks • Arm 2: brazikumab dose 2 IV on day 1, 15 and 43 + SC brazikumab from day 71 and every 4 weeks • Arm 3: brazikumab dose 3 IV on day 1, 15 and 43 + SC brazikumab from day 71 and every 4 weeks • Arm 4: vedolizumab 300 mg IV on day 1, 15 and 43 + IV vedolizumab from day 99 and every 8 weeks • Arm 5: placebo	Primary • Clinical remission at week 10 Secondary • Sustained clinical remission at week 10 and 54	• FPCD: Q3 2018 • Data anticipated: 2022+
Phase II **NCT04277546**	Ulcerative Colitis	300	• Open label extension	• Clinically significant adverse events	• FPCD: Q1 2020 • Data anticipated: 2022+

Nirsevimab (Respiratory syncytial virus mAb-YTE)

Infection

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT02878330**	29-35 WK GA (Gestational age) infants	1,453	• Randomised, double-blind, placebo-controlled trial • Route of administration: intramuscular	• 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 palivizumab	1,500	• Randomised, Double-blind, palivizumab-controlled • Route of administration: intramuscular Global trial – 32 countries	• Primary: Safety and tolerability • Secondary: PK, ADA and descriptive efficacy	• FPCD: Q3 2019 • LPCD: Q4 2020 • Data anticipated: H2 2021
Phase III **MELODY** **NCT03979313**	Healthy infants (born 35 weeks 0 days or greater GA)	3,000	• Randomised, Double-blind, placebo-controlled • Route of administration: intramuscular Global trial – 31 countries	• Primary: Efficacy • Secondary: Safety, PK, ADA	• FPCD: Q3 2019 • LPCD: Q3 2020 • Data readout: Q1 2021 • Primary endpoint met
Phase II **Japan IC** **NCT04484935**	Immunocompromised Japanese children who are ≤ 24 months of age at the time of dose administration	30	• Open-label, Uncontrolled, single-dose Study • Route of administration: intramuscular Japan only	• Primary: Safety and tolerability • Secondary: PK, ADA, efficacy	• FPCD: Q3 2020 • Data readout: 2022+

AZD1222 (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II **COV001 (UK)** **NCT04324606** **Partnered**	Healthy adults Age 18-55 years	1,077	Single-blinded, randomised, controlled, multi-centre trial • AZD1222 • Control vaccine: MenACWY UK	• Primary endpoint: efficacy and safety • Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q2 2020
Phase I/II **COV005 (SA)** **NCT04444674** **Partnered**	Healthy adults Age 18-65 years HIV+ subgroup	2,125	Adaptive, double-blinded, randomised placebo-controlled trial • AZD1222 • Placebo South Africa	• Primary endpoint: efficacy, safety, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q4 2020 • Data readout: Q1 2021
Phase II/III **COV002 (UK)** **NCT04400838** **Partnered**	Main efficacy trial: healthy adults aged ≥18 years Healthy adults 56 - <70 years Healthy adults ≥70 years Healthy children 5 – 12 years	10,812	Single-blinded, randomised, controlled, multi-centre trial with sequential age escalation/de-escalation immunogenicity sub-studies that include prime boost • AZD1222 • Control vaccine: MenACWY UK	• Primary endpoint: efficacy and safety • Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q4 2020 • Data readout: Q1 2021
Phase III **D8110C00001 (US, global)** **NCT04516746**	Healthy adults Age 18-65 years	32,429	Adaptive, double-blinded, randomised placebo-controlled trial • AZD1222 • Placebo US, with intent to expand to other countries	• Primary endpoints: efficacy, safety, tolerability, and reactogenicity • Secondary endpoints: immunogenicity	• FPCD: Q3 2020 • Data readout: Q1 2021
Phase III **COV003 (Brazil)** **NCT04536051** **Partnered**	Health professionals and adults with high potential for exposure to SARS-CoV-2 Age 18-55 years	10,414	Single-blinded, randomised, controlled multi-centre trial • AZD1222 • Control vaccine: MenACWY Brazil	• Primary endpoint: efficacy • Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity	• FPCD: Q2 2020 • LPCD: Q4 2020 • Data readout: Q1 2021
Phase III **D8111C00001** **NCT04540393**	Healthy adults Age ≥18 years	100	Open-label, non-comparative trial Russia	• Primary endpoints: safety, tolerability • Secondary endpoints: immunogenicity	• Paused
Phase I/II **D8111C00002** **NCT04568031**	Healthy adults Age ≥18 years	256	Double-blinded, randomised, placebo-controlled multi-centre trial • AZD1222 • Placebo Japan	• Primary endpoints: safety, tolerability, reactogenicity, immunogenicity • Secondary endpoints: immunogenicity	• FPCD: Q3 2020
Phase I/II **COV004 (Kenya)**	Healthy adults	400	Double-blinded, randomised, placebo-controlled multi-centre trial • AZD1222 • Control vaccine: rabies Kenya	• Primary endpoints: safety, tolerability, reactogenicity, immunogenicity • Secondary endpoints: immunogenicity	• FPCD: Q4 2020

AZD7442 (LAAB combination of AZD8895 & AZD1061)

Prevention and treatment of COVID-19

Trial	Population	Patients/Subjects	Design	Endpoints	Status
Phase I **NCT04507256**	Healthy adults Age 18-55 years	60	Double-blinded, randomised, placebo controlled, single ascending dose study AZD7442/placebo (10:2) Single center, UK	• Primary endpoint: safety, tolerability and PK • Secondary endpoints: immunogenicity	• FPFD: August 2020 • LPCD: October 2020
Phase III **PROVENT** **D8850C00002** **NCT04625725**	Adults having increased risk for inadequate response to active immunization or having increased risk for SARS-CoV-2 infection	5,000	Double-blinded, randomized, placebo controlled, multi center study to determine safety and efficacy AZD7442/placebo (2:1) Pre-exposure Countries: USA, UK, Belgium, France, Spain	• Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence of nucleocapsid antibodies, incidence of emergency visits, incidence of PCR positive, incidence of ADA to AZD7442 in serum and AZD7442 serum concentrations	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data anticipated: H2 2021
Phase III **STORM CHASER** **D8850C00003** **NCT04625972**	Adults with potential exposure To an identified individual with confirmed SARS-COV2 infection and at risk of developing COVID-19	1,125	Double-blinded, randomized, placebo controlled, multi center study to determine safety and efficacy AZD7442/placebo (2:1) Post-exposure Countries: USA and UK	• Primary endpoint: positive symptomatic illness post –dose • Secondary endpoints: Incidence of nucleocapsid antibodies, incidence of COVID-19 related death, incidence of all cause mortality, incidence of ADA to AZD7442 in serum and ZD7442 serum concentrations	• FPCD: Q4 2020 • LPCD: Q1 2021 • Data anticipated: Q2 2021
PHASE III **TACKLE** **NCT04723394**	Adults with confirmed mild to moderate SARS-COV2 infection. Symptomatic patients with documented positive SARS-Cov-2 molecular test.	1,700	Double-blinded, randomized, placebo controlled, multi center study to determine safety and efficacy of AZD7442 for treatment of Covid-19 in non-hospitalized patients AZD7442/placebo (1:1) Countries: UK, Germany, Spain, Italy, Hungary, Russia, US, Mexico, Japan, Poland, Czech Republic, Peru, Argentina, Brazil, Bulgaria 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	• FPCD: Q1 2021 • Data anticipated: Q2 2021

Other COVID-19 trials

Treatment of COVID-19

Trial	Compound	Population	Patients	Design	Endpoints	Status
Phase III DARE-19 NCT04350593	*Farxiga*	COVID-19	1,250	• Current SoC or current SoC + *Farxiga*	• Primary outcome measures: time to first occurrence of either death from any cause or new/worsened organ dysfunction through 30 days of follow up or improving clinical recovery; hierarchical composite outcome measures including time to death from any cause through day 30, new/worsened organ dysfunction, clinical status at day 30 and hospital discharge before day 30 and alive at day 30	• FPCD: Q2 2020 • LPCD: Q1 2021 • Data readout: Q1 2021 • Primary endpoint not met
Phase II TACTIC-E NCT04393246	*Farxiga*	COVID-19	1,407	• Current SoC or current SoC + *Farxiga* + ambrisentan	• Primary Outcome Measures: time to incidence of the composite endpoint of: death, mechanical ventilation, extracorporeal membrane oxygenation, cardiovascular organ support, or renal failure	• FPCD: Q4 2020 • Data anticipated: Q2 2021
Phase IIIa TACTIC-COVID NCT04355637	*Pulmicort*	COVID-19	300	• Current SoC or SoC + *Pulmicort*	• Primary outcome measures: proportion of patients in both arms fulfilling the criteria for treatment failure	• FPCD: Q2 2020 • Data anticipated: Q2 2021
Phase IIIa STOIC NCT04416399	*Pulmicort*	COVID-19	478	• Current SoC or SoC + *Pulmicort*	• Primary Outcome Measures: emergency department attendance of hospitalisation related to COVID-19	• FPCD: Q2 2020 • Data readout: Q1 2021
Phase IIIa INHASCO NCT04331054	*Symbicort*	COVID-19	436	• Current SoC or SoC + *Symbicort*	• Primary Outcome Measures: time (in days) to clinical improvement within 30 days after randomisation	• FPCD: Q2 2020 • Data anticipated: Q2 2021
Phase II ACCORD	MEDI3506	COVID-19	180	• Current SoC or current SoC + MEDI3506	• Primary endpoints: time to a 2-point improvement on a 9-point category ordinal scale, discharge from hospital, or considered fit for discharge whichever comes first by Day 29	• FPCD: Q2 2020 • Data anticipated: Q2 2021

BioPharmaceuticals – early-stage development

Cotadutide (GLP-1-glucagon agonist)

Diabetes/CKD, NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT03555994	Adults with type-2 diabetes	44	• Part A: cotadutide or placebo s.c. • Part B: cotadutide s.c. or placebo s.c. or liraglutide s.c. • Sweden, Netherlands, UK	• Primary: change in hepatic glycogen concentration postprandially, adjusted by liver volume • Secondary: safety • Secondary: tolerability • Secondary: immunogenicity	• FPCD: Q2 2018 • Part A LPCD: Q4 2018 • Data readout: Q1 2019 • Part B FPCD: Q1 2020 • LPCD: Q1 2021 • Data readout: H1 2021
Phase II NCT03596177	Overweight and obese patients with type-2 diabetes	27	• Cotadutide or placebo s.c. • UK	• Primary: efficacy body weight loss • Secondary: change in total energy intake • Secondary: change in total energy expenditure, active energy expenditure, resting energy expenditure • Secondary: safety	• FPCD: Q4 2018 • LPCD: Q4 2019 • Data readout: Q4 2020
Phase II NCT04019561	Obese patients with non-alcoholic fatty liver disease (NAFLD)/non-alcoholic steatohepatitis (NASH)	72	• Arm1: cotadutide high dose s.c. • Arm2: placebo high dose s.c. • Arm3: cotadutide low dose s.c. • Arm4: placebo low dose s.c. • US	• Primary: safety and tolerability • Secondary: change in hepatic fat fraction, • Secondary: change in liver fat volume • Secondary: change in visceral adipose tissue	• FPCD: Q4 2019 • Data Readout: H2 2021
Phase II NCT04515849	A Study of Cotadutide in participants who have chronic kidney disease with type 2 diabetes mellitus	225	• Arm1: cotadutide 100 micrograms • Arm2: cotadutide 300 micrograms • Arm3: cotadutide 600 micrograms • Arm4: semaglutide • Arm5: placebo	• Primary: efficacy change in UACR • Secondary: Change in HbA1c • Secondary: Change in glucose measured by CGM • Secondary: Effects on body weight • Secondary: Safety, tolerability, Immunogenicity	• FPCD: Q4 2020 • LPCD: Q2 2022 • Data readout: H2 2022
Phase I NCT04091373	Healthy adult patients	36		• Primary: to evaluate exposure following a single s.c of cotadutide at each of 3 different sites of injection • Secondary: immunogenicity • Secondary: safety and tolerability	• FPCD: Q4 2019 • LPCD: Q1 2020 • Data readout: Q4 2020

Verinurad (URAT1 inhibitor)

CKD, HFpEF

Trial	Population	Patients	Design	Endpoints	Status
Phase II SAPPHIRE **NCT03990363**	Patients with: • sUA ≥6.0 mg/dL • eGFR ≥25 mL/min/1.73 m2 Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI formula) • Mean UACR between 30 mg/g and 5000 mg/g	861	• Arm A; Verinurad 12 mg + allopurinol 300 mg • Arm B Verinurad 7.5 mg + allopurinol 300 mg • Arm C; Verinurad 3 mg to 24 mg + allopurinol 300 mg • Arm D; Verinurad placebo + allopurinol 300 mg • Arm E; Verinurad placebo + allopurinol placebo This trial is multi-centre trial conducted in USA, China, Czech Republic, France, Hungary, Israel, Italy, Mexico, Poland, Romania, Slovakia, South Africa, Spain	Ratio of urinary albumin to urinary creatinine Changes in eGFR, Cystatin C, and uric acid	• FPCD: Q3 2019 • Data anticipated: 2022
Phase I **NCT03118739**	Healthy volunteers	24	• Treatment A: Verinurad 24 mg ER8 formulation + 300 mg • allopurinol • Treatment B: Verinurad 40 mg IR formulation + 300 mg • allopurinol • Treatment C: Matched placebos for both verinurad and allopurinol The trial is a single-centre, randomised, placebo-controlled, double-blind, 3-period, cross-over trial conducted in Germany	To assess the effect of a single dose of verinurad given as either a 24 mg extended-release (ER8) formulation (therapeutic exposure) or a 40 mg immediate-release (IR) formulation (supra-therapeutic exposure), both in combination with allopurinol 300 mg, on the QT interval corrected for heart rate using Fridericia's formula (QTcF) compared to placebo	• FPCD: Q3 2020 • Data readout: H2 2020
Phase I **NCT04532918**	Healthy volunteers	14	• Treatment A: Verinurad 7.5 mg ER8 formulation + 300 mg Allopurinol under fasted conditions • Treatment B: Verinurad 7.5 mg IR formulation + 300 mg allopurinol + cyclosporin 600 mg under fasted conditions • Treatment C: Verinurad 7.5 mg IR formulation + 300 mg allopurinol + rifampicin 600 mg under fasted conditions The trial is a single-centre, randomised, open-label, 3-period, fixed sequence, trial conducted in Germany	To quantify the effects of cyclosporine, a broad transporter inhibitor, and rifampicin, an OATP1B1/3 inhibitor,on verinurad pharmacokinetics (PK).	• FPCD: Q3 2020 • LPCD: Q4 2020 • Data anticipated: H1 2021
Phase II AMETHYST **NCT04327024**	Patients with heart failure with preserved ejection fraction	435	• Arm A: verinurad 12 mg and 24 mg + allopurinol 300 mg • Arm B: verinurad placebo + allopurinol 300 mg • Arm C: verinurad placebo + allopurinol placebo The trial is a multi-centre trial conducted in Argentina, Australia, Austria, Bulgaria, Canada, Germany, Mexico, Poland, Russia, Slovakia South Korea, USA	Peak V02 Change from baseline at Week 28 in exercise capacity Change from baseline at Week 28 in Kansas-City Cardiomyopathy Questionnaire-Total Symptom Score (KCCQ-TSS)	• FPCD: Q3 2020 • Data readout: 2022+

AZD2373

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04269031**	Healthy volunteers	48	SAD Dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort Trial conducted in the US	Primary: • Safety and tolerability Secondary; • PK parameters	• FPCD: Q1 2020

AZD2693 (resolution of NASH)

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04142424**	Healthy volunteers	48	SAD 6 cohorts with 6 volunteers receiving AZD2693 and 2 volunteers receiving placebo in each cohort Route of administration: subcutaneous injections Trial conducted in the US.	Primary: • Safety and tolerability Secondary; • PK	• FPCD: Q4 2019 • Data anticipated: H2 2021
Phase I **NCT04142424**	NAFLD F0-F3	60	MAD 3 cohorts receiving AZD2693 and placebo in each cohort Route of administration: subcutaneous injections Trial conducted in the US.	Primary: • Safety and tolerability Secondary; • PK	• FPCD: Q1 2021 • Data anticipated: H1 2021

AZD3427 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04630067**	SAD – Healthy Volunteers MAD – Heart Failure	104	• Multi-center single and multiple ascending dose study (SAD and MAD) planned in 96 participants (US) • Part A (SAD) will include 6 cohorts randomized to AZD3427 or placebo • Part B (MAD) will include cohorts randomized to AZD3427 or placebo	Safety & Tolerability	• FPCD: Q4 2020 • Data anticipated: 2022+

AZD3366

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04588727**	Healthy volunteers	79	SAD Part A Dose escalation in 6 cohorts with 6 volunteers receiving AZD3366 and 2 volunteers receiving placebo in each cohort Part B 12 subjects receiving AZD3366 and ticagrelor and ASA Trial conducted in the US	Primary: • Safety and tolerability Secondary; • PK parameters	• FPCD: Q4 2020 • LPCD: H2 2021

MEDI3506 (IL33 ligand mAb)

Diabetic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04170543**	Adult patients with diabetic kidney disease	565	• Arm A- MEDI3506 Dose 1 + dapagliflozin • Arm B- MEDI3506 Dose 2 + dapagliflozin • Arm C- MEDI3506 Dose 3 + dapagliflozin • Arm D- MEDI3506 Dose 4 + dapagliflozin • Arm E- placebo + dapagliflozin This trial is multi-centre trial conducted in USA, Canada, Japan and additional countries.	• Efficacy and safety	• FPCD: Q4 2019

AZD4831 (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02712372**	Healthy patients	c. 96	SAD trial (one trial site in Germany) • Planned to investigate 6 different dose levels vs. placebo but up to 10 cohort may be used	• Safety and tolerability • PK parameters	• FPCD: Q3 2016 • LPCD: Q4 2016 • Data readout Q2 2017
Phase I **NCT03136991**	Healthy patients	c. 40	MAD (one trial site in USA) • The planned number of cohorts is four but up to five cohorts may be included	• Safety and tolerability • PK parameters	• FPCD: Q2 2017 • LPCD: Q4 2017 • Data readout: Q1 2018
Phase IIa **NCT03756285**	HFpEF	96	Arm 1: AZD4831 Arm 2: placebo Global trial – five countries	• Primary endpoint: The change from baseline in MPO activity in % after AZD4831 treatment	• FPCD: Q4 2018 • Data readout: Q2 2021
Phase I **NCT04232345**	Healthy patients	32	MAD trial in Japanese and Chinese patients	• Safety and tolerability	• FPCD Q1 2020 • Data anticipated: Q2 2021
Phase I NCT04407091	Healthy patients	6	hADME (one trial site in UK) • Oral administration • Open-label trial to characterize the absorption, distribution, metabolism and excretion following a single oral dose of [14C]AZD4831 in healthy male volunteers	• Mass balance, with routes and rates of elimination of [14C]AZD4831. • Metabolite profiling and structural identification • PK and total radioactivity	• FPCD: Q2 2020 • LPCD: Q3 2020 • Data anticipated: Q2 2021

AZD5718 (FLAP inhibitor)

Cardiovascular disease & Chronic Kidney Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **NCT03317002**	CAD	129	• Arm 1: AZD5718 Dose A • Arm 2: AZD5718 Dose B • Arm 3: placebo Global trial – three countries in Europe	• Primary endpoint: PD effect of AZD5718 by assessment of u-LTE4	• FPCD: Q4 2017 • LPCD: Q4 2019 • Data readout: Q1 2021
Phase I **NCT03948451**	Healthy patients	6	hADME trial (one trial site in UK) • Oral administration Open-label trial to characterize the absorption, distribution, metabolism and excretion following a single oral dose of [14C]AZD5718 in healthy male volunteers	• Mass balance, with routes and rates of elimination of [14C]AZD5718. • Metabolite profiling and structural identification • PK and total radioactivity	• FPCD: Q2 2019 • LPCD: Q2 2019
Phase I **NCT04087187**	Healthy patients	14	BA trial (one trial site in UK) An open-label, randomized, 3-period, 3-treatment, crossover trial to assess the drug absorption into the blood after administration of 3 doses of AZD5718	• To evaluate the pharmacokinetics and exposure of 3 different doses of AZD5718 • Safety and tolerability	• FPCD: Q4 2019 • LPCD: Q4 2019
Phase I **NCT04210388**	Healthy patients	12	BA trial (one trial site in UK) The trial is a randomized, single-dose, open-label, combined 2x2 dose and 3x3 dose crossover design in fixed sequence.	To evaluate: • The relative bioavailability of different formulations • Safety and tolerability	• FPCD: Q1 2020 • LPCD: Q1 2020
Phase I **NCT0473275**	Healthy patients	16	BA trial (one trial site in UK) The trial is a randomized, open-label, 3-period, single dose, crossover study in healthy subjects.	To evaluate • The relative bioavailability of different formulations • Safety and tolerability	• FPCD: Q1 2021 • LPCD: Q1 2021
Phase IIb **NCT04492722**	CKD	632	Interventional A Phase IIb randomised, double-blind, placebo-controlled, multi-centre, dose-ranging trial of AZD5718 in participants with proteinuric CKD	To evaluate: • dose-response efficacy • Safety • pharmacokinetics (PK)	• FPCD: Q4 2020

AZD8233 (PCSK9 inhibitor, sub-cutaneious)

Dyslipidemia

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03593785**	Healthy subjects	72	SAD 7 cohorts with 6 subjects receiving AZD8233 and 2 subjects receiving placebo in each cohort Trial conducted in the US.	Primary: • Safety and tolerability Secondary; • PK and PD parameters	• FPCD: Q3 2018 • LPCD: Q3 2019
Phase I **NCT04155645**	Dyslipidemia	33	MAD Up to 3 cohorts with 8 subjects receiving AZD8233 and 3 subjects receiving placebo in each cohort Trial conducted in the US	Primary: • Safety and tolerability Secondary; • PK and PD parameters	• FPCD: Q1 2020 • LPCD: Q4 2020
Phase II **NCT04641299**	Dyslipidemia	108	Subjects are randomized across four different treatment arms in a 1:1:1:1 ratio for a 12-week treatment period Arm 1: High AZD8233 dose Arm 2: Medium AZD8233 dose Arm 3: Low AZD8233 dose Arm 4: placebo Trial conducted in 3 countries (US, Slovakia and Denmark)	Primary: • Efficacy	• FPCD: Q4 2020 • LPCD: Q1 2021
Phase II **NCT04823611**	Dyslipidemia	91	Study consists of Part A and Part B, both with a 12-week treatment period Part A: 11 subjects randomized in an 8:3 ratio into 1 of the 2 treatment arms; AZD8233 high dose or placebo Part B: 80 subjects randomized across four different treatment arms in a 1:1:1:1 Arm 1: High AZD8233 dose Arm 2: Medium AZD8233 dose Arm 3: Low AZD8233 dose Arm 4: placebo Trial conducted in Japan	Primary in Part A: • Safety and tolerability Primary in Part B: • Efficacy	• FPCD: Q1 2021

MEDI8367

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04365218**	Healthy volunteers CKD	70	Single ascending dose 6 cohorts Arm 1: MEDI8367 Arm 2: placebo Subcutaneous administration Trial conducted in the US	Primary: • Safety and tolerability Secondary; • PK parameters • ADA	• FPCD: Q3 2020

AZD8601 (VEGF-A modified RNA)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02935712**	Type 2 diabetic patients	c. 60	SAD trial (one trial site in Germany) • Planned to investigate 3 different dose levels vs. placebo but up to 5 cohort may be used	• Safety and tolerability	• FPCD: Q1 2017 • LPCD: Q3 2017 • Data readout: Q1 2018
Phase IIa **NCTT03370887**	HF	Up to 33	Phase IIa trial (two trial sites in Finland, two in Germany) • Arm 1: AZD8601 Dose A • Arm 2: AZD 8601 Dose B • Arm 3: placebo	• Safety and tolerability	• FPCD: Q1 2018

AZD9977

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03435276**	Healthy volunteers	27	MAD Dose escalation in 3 cohorts with 6 subjects receiving AZD9977 and 3 volunteers receiving placebo in each cohort Trial conducted in the UK.	Primary: • Safety and tolerability Secondary; • PK parameters	• FPCD: Q1 2018 • LPCD: Q2 2018 • Data readout: Q3 2018
Phase I **NCT03450759**	Healthy volunteers	12	Bioavailability trial Investigation of four different oral formulations of AZD9977 and influence of food. Trial conducted in the UK.	Primary: • relative bioavailability vs. oral suspension (reference) • PK parameters	• FPCD: Q2 2018 • LPCD: Q2 2018 • Data readout: Q3 2018
Phase I **NCT03682497**	HF	60	Proof of differentiation To compare the effect of AZD9977 with spironolactone on serum potassium	Primary: • serum potassium	• FPCD Q4 2018 • LPCD Q1 2019
Phase I **NCT03843060**	Healthy volunteers	14	DDI To assess the effect of itraconazole on the pharmacokinetics of AZD9977 Trial conducted in the US	Primary: • PK parameters Secondary; • Safety and tolerability	• FPCD: Q1 2019 • LPCD: Q1 2019 • Data readout: Q3 2019
Phase I **NCT03801967**	Healthy volunteers	45	JSMAD Single and multiple-ascending dose administration in Japanese healthy volunteers. Trial conducted in the UK	Primary: • Safety and tolerability Secondary; • PK parameters	• FPCD: Q1 2019 • LPCD: Q2 2019 • Data readout: Q3 2019
Phase I **NCT03804645**	Healthy volunteers	12	Bioavailability trial Investigation of four different oral formulations of AZD9977 and influence of food. Trial conducted in the UK	Primary: • relative bioavailability vs. capsule formulation (reference) • PK parameters	• FPCD: Q1 2019 • LPCD: Q2 2019 • Data readout: Q3 2019
Phase I **NCT04469907**	Renal Impairment	32	Renal Impairment Single dose administration of AZD9977 conducted in participants with severe renal impairment and compared with matched participants with normal renal function Trial conducted in the US	Primary: • PK parameters Secondary: • Safety and tolerability	• FPCD: Q3 2020 • LPCD: H1 2021 • Data readout: H2 2021
Phase I **NCT04686591**	Healthy volunteers	8	ADME Study of absorption-distribution-metabolism-excretion (ADME) of ^{14}C-AZD9977 following a single oral dose and absolute bioavailability of a single oral dose with respect to AZD9977 Trial conducted in the UK	Primary: • Absolute bioavailability • The mass balance, rates and routes of • elimination Secondary: • Safety and tolerability	• FPCD: Q1 2021 • LPCD: Q1 2021 • Data readout: H2 2021

Zibotentan (endothelin receptor antagonist)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb **NCT04724837**	Chronic Kidney Disease	660	Global recruitment Part A: 132 participants equally randomised across 4 arms: Arm 1: Zibotentan dose A + *Farxiga* 10 mg once daily. Arm 2: Zibotentan dose A once daily. Arm 3: *Farxiga* 10 mg once daily. Arm 4: Placebo once daily. Part B: 528 participants equally randomised across 6 arms: Arm 1: Zibotentan dose C + *Farxiga* 10 mg once daily. Arm 2: Zibotentan dose B + *Farxiga* 10 mg once daily. Arm 3: Zibotentan dose A + *Farxiga* 10 mg once daily. Arm 4: Zibotentan dose A once daily. Arm 5: *Farxiga* 10 mg once daily. Arm 6: Placebo once daily.	Primary Endpoint: Change in log-transformed UACR from baseline to week 12. Secondary Endpoints: •Change in log-transformed UACR from baseline to week 12. •Change in blood pressure from baseline (Visit 2) to week 12. •The least squares mean change of UACR at week 12 from the 3 Zibo/Dapa dose groups and the dapagliflozin monotherapy group. •Change in eGFR from baseline to week 1, week 12 and week 14. •Change in eGFR from week 1 to week 12.	• Initiating

Biologics

Cardiovascular & metabolic diseases

Trial	Compound	Population	Patients	Design	Endpoints	Status
Phase IIa **NCT03351738**	MEDI5884 cholesterol modulation	Adults with stable CHD	133	• MEDI5884 (5 dose cohorts) vs. placebo in stable CHD patients	• Safety profile in terms of AEs, vital signs, ECG, lab variables • Changes in HDL-C over time • PK, immunogenicity, and Apolipoprotein B	• FPCD: Q4 2017 • Data readout: Q4 2018
Phase I **NCT03654313**	MEDI6570	Atherosclerotic cardiovascular disease	88	• SAD followed by multi ascending dose with 3 monthly doses in T2DM subjects	• Primary endpoints: Safety and tolerability	• FPCD: Q4 2018 • LPCD: Q3 2020 • Data readout: Q4 2021
Phase IIb **NCT04610892**	MEDI6570	Post MI	792	Evaluation of anti-inflammatory potential of MEDI6570 and its effect on surrogates for atherosclerotic and heart failure (HF) events . Subjects are randomized across four different treatment arms in a 1:1:1:1 ratio Arm 1: High AZD6570 dose Arm 2: Medium AZD6570 dose Arm 3: Low AZD6570 dose Arm 4: Placebo Trial conducted in 9 countries (US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland,)	• Efficacy and safety	• FPCD: Q4 2020 • Data anticipated: 2022+

AZD0449 (inhaled JAK-1 inhibitor)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03766399**	Healthy subjects and patients with mild asthma	156	SAD/MAD/Bridge trial (UK) Part 1 SAD • Dose escalation in 6 cohorts with 6 subjects receiving AZD0449 and 2 subjects receiving placebo in each cohort • i.v. cohort with 2x6 subjects Part 2 MAD: • 2 cohorts of (6, 6,) mild asthmatics receiving two different doses of AZD0449 and (3,3) patients receiving placebo in each cohort • 1 cohort of 6 patients receiving 1 dose of AZD0449 and 2 patients receiving placebo Part 3 bridge • 1 cohort of 6 patients receiving 1 dose of AZD0449 (DPI formulation) and 2 patients receiving placebo. • Up to 18 mild asthmatic patients will receive AZD0449 (DPI) and 18 patients receiving placebo. Interim analysis planned after 9 +9 patients. Trial conducted in the UK	Primary endpoint: • Safety and tolerability Secondary endpoint: • PK parameters • FENO	• FPCD: Q4 2018 • Data anticipated: H1 2021

AZD1402 (IL4 receptor alpha antagonist)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib **NCT03574805** **Partnered**	Patients with mild asthma	84	PoM. A dose-escalating, single blind trial to assess the safety, tolerability, and pharmacokinetics of multiple doses of PRS-060 administered by oral Inhalation In subjects with mild asthma Australia	Primary endpoint: • Safety and tolerability Secondary endpoint: • PK parameters • Potential immunogenicity • Change in FENO	• LPCD: Q3 2018

MEDI3506 (IL33 ligand mAb)

COPD, atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II **NCT04212169**	Adult subjects with atopic dermatitis	152	Randomised, blinded, placebo-controlled trial to determine the efficacy and safety of three different doses of MEDI3506 by SC route vs placebo Conducted in US, Australia, Germany, Poland, UK & Spain	• Primary: change from baseline at week 16 in Eczema Area and Severity Index (EASI) score • Secondary: safety and other efficacy measures	• FPCD: Q4 2019
Phase II **NCT04570657**	Adult participants with uncontrolled moderate to severe asthma	228	Randomised, double-blind, placebo-controlled trial to evaluate the efficacy, safety, pharmacokinetics (PK) and immunogenicity of two different doses of MEDI3506 by SC route vs placebo Conducted in US, Argentina, Germany, Hungary, Poland & South Africa	• Primary: change from baseline at week 16 in FEV1 • Secondary: safety and other efficacy measures	• FPCD: Q4 2020
Phase II **NCT04631016**	Adult subjects COPD and chronic bronchitis	322	Randomised, double-blind, placebo-controlled, parallel group, proof of concept trial to evaluate the efficacy and safety of MEDI3506 as a single dose by SC route versus placebo Conducted in US, Canada, Denmark, Germany, Hungary, Netherlands, Poland, South Africa, Spain, Australia & UK	• Primary: change from baseline to week 12 in FEV1 • Secondary: safety and other efficacy measures	• FPCD: Q1 2021

AZD7986 (DPP1)

COPD

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02653872**	Healthy volunteers	15	This is a phase I, non-randomised, fixed sequence, 3-period, drug-drug interaction trial to assess the PK of AZD7986 in healthy subjects when administered alone and in combination with multiple doses of verapamil and itraconazole or diltiazem • Arm 1: AZD7986 (alone) treatment period 1 • Arm 2: verapamil (with AZD7986) treatment period 2 • Arm 3: itraconazole (with AZD7986) treatment Period 3 • Arm 4: diltiazem (with AZD7986) treatment period 3	• Safety and tolerability • PK/PD and DDI	• FPCD: Q1 2016 • Data readout: Q2 2016
Phase I **NCT02303574**	Healthy volunteers	89	A phase I, randomised, single-blind, placebo-controlled, 2-part trial to assess the safety, tolerability, PK and food effect of single and multiple oral doses of AZD7986 in healthy volunteers. • Arm 1: AZD7986, single and multiple oral doses • Arm 2: placebo, single and multiple doses	• Safety and tolerability • PK/PD • Bioavailability	• FPCD: Q4 2014 • Data readout: Q3 2016

AZD8871 (MABA, inhaled)

Respiratory

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa **NCT03645434**	Patients with COPD	73	Randomised, double-blind, placebo and active-controlled crossover trial. Eligible patients will be randomised in 1:1:1:1:1:1:1 ratio to 1 of 6 treatment sequences and will receive 1 of the following 3 treatments sequence in the form of dry powder inhalation: • AZD8871 600 µg once daily • Anoro® Ellipta® (55 µg umeclidinium [UMEC]/ 22 µg vilanterol [VI]) once daily • Placebo	Primary endpoint: • Change from baseline in trough FEV_1 on day 15 Secondary endpoints: • To characterize the pharmacokinetics of AZD8871 following multiple inhaled doses • To assess safety and tolerability of AZD8871	• FPCD: Q4 2018 • LPCD: Q2 2019 • Data readout: Q3 2019

AZD9567 (SGRM, oral)

Respiratory

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02760316**	Healthy subjects	71	MAD trial with a total of 6 dose levels of AZD9567: 10 mg, 20mg, 40mg, 80mg and 125 mg as well as with 3 dose levels of prednisolone: 5 mg, 20 mg and 40 mg	Primary endpoint: • To assess the safety and tolerability of AZD9567 following multiple oral ascending doses in subjects with BMI between 28 and 38 kg/m2 and with a positive glucose tolerance test (7,8 to 11,0 mmol/L)	• FPCD: Q2 2016 • Data readout: Q2 2018
Phase IIa **NCT03368235**	Patients with active RA	21	A randomised, double-blind, parallel trial to assess the efficacy, safety and tolerability of AZD9567 compared to prednisolone 20 mg in patients with active rheumatoid arthritis	Primary endpoint: To assess the efficacy of AZD9567, 40 mg, compared to prednisolone 20 mg in patients with active RA in spite of stable treatment with conventional and/or s.c./i.v. biological DMARDs (Disease-modifying antirheumatic drugs)	• FPCD: Q1 2018 • Data readout: Q2 2020
Phase II **NCT04556760**	Patients With Type 2 Diabetes Mellitus	42	A study to assess the effect on glycaemic control of AZD9567, as measured by the glucose AUC(0-4) versus baseline following a standardised mixed meal tolerance test (MMTT), compared to prednisolone in adults with type 2 diabetes mellitus (T2DM). The study will also evaluate the safety, tolerability, and pharmacokinetics (PK) of AZD9567	Primary endpoint: • Change in glucose AUC(0-4) versus baseline compared to prednisolone following a standardised MMTT	• FPCD: Q4 2020 • LPCD: Q2 2021 • Data readout: H2 2021

AZD0284 (RORγ inverse agonist)

Plaque psoriasis vulgaris

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02976831**	Healthy subjects	80	Part 1 (SAD) • Seven different dose levels investigated vs. placebo • Oral administration	• Safety and tolerability and PK following oral administration with single ascending dose • Preliminary assessment of the effect of food on the single dose PK parameters of AZD0284	• FPCD: Q3 2016 • LPCD: Q2 2017
			Part 2 (MAD) • Three different dose levels investigated vs. placebo in healthy subjects • Oral administration	• Safety and tolerability & PK in healthy subjects following administration of multiple ascending oral doses • PoM confirmed by demonstrating that oral dosing of AZD0284 reduces IL-17 secretion by ex vivo stimulated whole blood T cells	• FPCD: Q1 2017 • LPCD: Q1 2017
Phase I **NCT03029741**	Healthy subjects	6	A single centre, open-label, non-randomised, single dose trial performed in 6 healthy male subjects aged 18 to 65 years, inclusive. The trial will assess the absolute bioavailability of a single oral dose of AZD0284 and the pharmacokinetics (PK) of a single intravenous (IV) microdose of [14C] AZD0284 in healthy male and female subjects. Oral AZD0284 and [14C] AZD0284 intravenous solution are referred to as the investigational products in this trial	• Determination of absolute bioavailability of AZD0284 • Safety and tolerability of AZD0284	• FPCD: Q1 2017 • LPCD: Q1 2017
Phase Ib **NCT03310320**	Patients with moderate to severe plaque psoriasis	15	This was a randomised, double-blind, placebo-controlled, multi-centre, parallel group Phase Ib study, designed to evaluate the pharmacodynamic (PD) effects, clinical efficacy and safety of AZD0284 compared with placebo as measured by the relative change from baseline in Psoriasis Area and Severity Index (PASI) score and biomarkers associated with the mechanism of disease and AZD0284.	• Relative change from baseline of IL-17A and CCL20 mRNA expression levels in lesional skin at Week 4. • Percent improvement from baseline in individual PASI score at Week 4	• FPCD: Q4 2017 • LPCD: Q1 2018

MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02508155**	Painful osteoarthritis of the knee	64 (healthy volunteers)	• SAD • Up to 8 i.v. cohorts are planned vs. placebo • 1 s.c. cohortis planned vs. placebo Europe only	• Safety, tolerability and PK	• FPCD: Q4 2019 • Data anticipated: H2 2021

MEDI1341 (alpha-synuclein mAb)

Parkinson's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT03272165**	Healthy volunteers	48	• SAD • Up to 6 i.v. cohorts are planned vs. placebo US only	• Safety, tolerability, PK, PD	• FPCD: Q4 2017 • LPCD: Q4 2020 • Data anticipated: Q2 2021
Phase I **NCT04449484**	Parkinson's Disease	36	• MAD • Up to 3 i.v. cohorts are planned vs placebo • US only	• Safety, tolerability, PK, PD	• FPCD: Q3 2020 • Data anticipated: 2022+

AZD4041 (orexin 1 receptor antagonist)

Opioid use disorder

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT04076540** **Partnered with Eolas Therapeutics Inc and NIH.**	Healthy volunteers	48 healthy volunteers	• Randomised, double blind, single ascending dose • Up to 6 cohorts are planned vs. placebo Single centre in US only	• Safety, tolerability, PK, PD	• FPCD: Q4 2019 • Data anticipated: H1 2021

MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase I **NCT02508155**	Painful osteoarthritis of the knee	160	• SAD & MAD • Up to 12 i.v. cohorts are planned vs. placebo • 1 s.c. cohorts are planned vs. placebo Europe only	• Safety, tolerability, PK, PD	• FPCD: Q1 2016 • LPCD: Q4 2020 • Data anticipated: Q2 2021
Phase II **NCT03755934**	Painful diabetic neuropathy	271	• Multiple dose trial • Up to 4 i.v. cohorts are planned vs. placebo Europe only	• Dose response, safety, tolerability, PK, PD	• FPCD: Q4 2018 • Data anticipated: H2 2021
Phase IIb **NCT04675034**	Painful osteoarthritis of the knee	300	• Multiple dose trial • 3 active s.c. dose cohorts vs. placebo Global (8 countries)	• Dose response, safety, tolerability, PK, PD, ADA	• FPCD: Q1 2021 • Data anticipated: H1 2022
Phase I **NCT04770428**	Healthy volunteers Japanese and Caucasian	20	• Multiple dose trial • 1 active s.c. dose cohort vs. placebo Europe only	• Safety, tolerability, PK, PD, ADA	• FPCD: Q2 2021 • Data anticipated: H2 2021

Other biologics

Infections

Trial	Compound	Population	Patients	Design	Endpoints	Status
Phase II **EudraCT 2014-001097-34**	Anti-Staph AT (suvratoxumab, MEDI4893)	Intubated ICU	213	• Placebo-controlled, single-dose, dose-ranging • Route of administration: intravenous	• Efficacy and safety	• FPCD: Q4 2014 • Data readout: Q4 2018
Phase II **NCT02696902**	Anti-Pseudomonas A mAb (MEDI3902)	Intubated ICU	195	• Placebo-controlled, single-dose, dose-ranging • Route of administration: intravenous	• Efficacy and safety	• FPCD: Q2 2016 • Data readout: Q4 2020

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
AI	Auto-injector
AKT	Protein kinase B
ALK	Anaplastic large-cell lymphoma kinase
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
AUC	Area under 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
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
CCL20	Chemokine (C-C motif) ligand 20
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
DoR	Duration of response
DPI	Dry powder inhaler
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
ESR	Externally sponsored trial
ESR1	Oestrogen receptor 1
ESSC	Esophageal squamous cell carcinoma
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FGFR	Fibroblast growth factor receptor
FLAP	5-lipoxygenase-activating protein
FPDC	First patient commenced dosing
FPG	Fasting plasma glucose
GA	Gestational age
GBM	Glioblastoma
gBRCAm or tBRCAm	Germline or tumour BRCA mutation somatic
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
HAI	Haemagglutination-inhibition
HbA1c	Hemoglobin 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
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
IL	Interleukin
i.m.	Intramuscular
IRC	Independent review committee
ISS	Investigator-sponsored studies
i.v.	Intraveneous
J-SD	Japanese single dose
Ki67	Protein that is encoded by the MKI67 gene in human
LAAB	Long acting antibody

List of abbreviations

LABA	Long acting beta agonist
LAMA	Long acting muscarinic agonist
LCAT	Lecithin-cholesterol acyltransferase
LCM	Lifecycle management
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 carcinoma
MD	Medium dose
MDI	Metered-dose inhaler
MDS	Myelodysplastic syndrome
MEK	Mitogen-activated protein kinase
MET	Tyrosine-protein kinase Met
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
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
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
RT	Radiation therapy
SABA	Short-acting beta2-agonist
SAD	Single ascending dose
SAE	Serious adverse event
SBRT	Stereotactic body radiation therapy
s.c.	Subcutaneous
SCLC	Small cell lung cancer
SD	Stable disease
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 physicians 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
TID	Ter in die (three times a day)
TJC	Tender joint count
TKI	Tyrosine kinase Inhibitor
TLR	Toll-like receptor 9
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
VEGF	Vascular endothelial growth factor
YTE	Triple-amino-acid (M252Y/S254T/T256E [YTE]) substitution

AstraZeneca

Clinical trials appendix

Q1 2021 results update