



Clinical trials appendix

FY 2021 results update



Movement since Q3 2021 update

New to Phase I	New to Phase II	New to Pivotal trial	New to registration
<p>NME AZD5055 Porcupine inhibitor idiopathic pulmonary fibrosis</p> <p>AZD7503 ASO non-alcoholic steatohepatitis</p> <p>AZD8205 B7-H4 TOP1i ADC solid tumours</p> <p>AZD8630[#] Inhaled TSLP mAb asthma</p> <p>AZD8701[#] + Imfinzi FOXP3 + PD-L1 solid tumours</p>	<p>NME AZD0171 + Imfinzi + CTx anti-LIF mAb + PD-L1 mAb + CTx 1st-line metastatic pancreatic ductal adenocarcinoma</p> <p>AZD4573 + Calquence CDK9 inhibitor + BTK inhibitor haematological malignancies</p>	<p>NME eplontersen[#] ligand-conjugated antisense patients with hereditary transthyretin-mediated amyloid polyneuropathy</p> <p>Additional indication datopotamab deruxtecan[#] TROPION-Breast01 TROP-2 targeting antibody drug conjugate 2-3 line HR+ HER2-breast cancer</p> <p>eplontersen[#] ligand-conjugated antisense patients with hereditary or wild-type transthyretin-mediated amyloid cardiomyopathy</p> <p>Lifecycle management Enhertu[#] DESTINY-Breast11 HER2 targeting antibody drug conjugate Neoadjuvant HER2-positive breast cancer</p> <p>Enhertu[#] DESTINY-Lung04 HER2 targeting antibody drug conjugate 1st-line non-small cell lung cancer</p> <p>Saphnelo[#] AZALEA (China) Type 1 IFN receptor mAb systemic lupus erythematosus</p> <p>Ultomiris (ALXN1210)[¶] anti-complement C5 mAb dermatomyositis</p>	<p>NME Imfinzi +/- tremelimumab + CTx POSEIDON [US, EU & JP]¹ PD-L1 mAb +/- CTLA-4 mAb + CTx 1st-line non-small cell lung cancer</p> <p>Lifecycle management Enhertu[#] DESTINY-Breast03 [US, EU & JP]¹ HER2 targeting antibody drug conjugate HER2-positive, unresectable and/or metastatic breast cancer subjects previously treated with trastuzumab and taxane</p> <p>Lynparza[#] OlympiA [US, EU & JP]¹ PARP inhibitor gBRCA adjuvant breast cancer</p> <p>Lynparza + abiraterone PROpel [EU]¹ PARP inhibitor + NHA prostate cancer</p> <p>Ultomiris (ALXN1210) [US]¹ anti-complement C5 mAb generalised myasthenia gravis</p> <p>Ultomiris (ALXN1210) [US]¹ anti-complement C5 mAb subcutaneous administration paroxysmal nocturnal haemoglobinuria and atypical haemolytic uraemic syndrome</p>



Movement since Q3 2021 update

Removed from Phase I	Removed from Phase II	Removed from Phase III	Removed from registration
<p><u>NME</u> AZD0284 RORg psoriasis/respiratory</p> <p>AZD0449 Inhaled JAK inhibitor asthma</p> <p>ALXN1830 anti-FcRn mAb Warm Autoimmune Haemolytic Anaemia</p>	<p><u>NME</u> AZD2811 nanoparticle Aurora B inhibitor solid tumours</p> <p>MEDI5884³ cholesterol modulation cardiovascular disease</p> <p>suvratoxumab³ mAb binding to <i>S. aureus</i> toxin prevention of nosocomial <i>Staphylococcus aureus</i> pneumonia</p>	<p><u>NME</u> AZD2816 (next generation COVID-19 vaccine) SARS-CoV-2 prevention of COVID-19</p>	<p><u>NME</u> Evusheld (AZD7442) [US EUA]² COVID-19 LAAB combination prevention and treatment of COVID-19</p> <p>Tezspire# (tezepelumab) NAVIGATOR [US]² TSLP mAb severe uncontrolled asthma</p>



Q4 2021 Oncology new molecular entity¹ pipeline

Phase 1 15 New Molecular Entities		Phase 2 17 New Molecular Entities		Phase 3 15 New Molecular Entities	
AZD0466# BCL2/xL haematological tumours	AZD1390 ATM glioblastoma	adavosertib# Wee1 ovarian / uterine serous / solid tumours	AZD5305 PARP1 solid tumours	camizestrant+CDK4/6i SERENA-6 SERD+CDK4/6 1L HR+ HER2- ESR1m breast cancer	camizestrant+palbociclib SERENA-4 SERD+CDK4/6 1L HR+ HER2- breast cancer
AZD2936# PD1/TIGIT bispecific mAb solid tumours	AZD4573 CDK9 haematological malignancies	AZD0171 + <i>Imfinzi</i> + CTx anti-LIF + PD-L1 + CTx 1L metastatic pancreatic ductal adenocarcinoma	cavivasertib# AKT prostate	cavivasertib#+abiraterone CAPItello-281 AKT+abiraterone PTEN deficient mHSPC	cavivasertib#+chemo CAPItello-290 AKT+chemo mTNBC 1L
AZD5991 MCL1 haematological malignancies	AZD7648# DNAPK solid and haematological tumours	AZD4573 + <i>Calquence</i> CDK9+BTK haematological malignancies	<i>Imfinzi</i> (platform) HUDSON PD-L1+multiple novel ONC therapies post IO NSCLC	cavivasertib#+fulvestrant + palbociclib CAPItello-292 AKT+fulvestrant+CDK4/6 HR+ breast 1L	cavivasertib#+fulvestrant CAPItello-291 AKT+fulvestrant locally-advanced (inoperable) or metastatic breast
AZD7789 PD1/TIM3 bispecific mAb solid tumours	AZD8205 B7-H4 TOP1i ADC solid tumours	camizestrant (AZD9833) SERD ER+ breast	<i>Imfinzi</i> # (platform) NeoCOAST PD-L1+multiple novel ONC therapies NSCLC	datopotamab deruxtecans# TROPION-Breast01 TROP2 2-3L HR+ HER2- BC	datopotamab deruxtecans# TROPION-Lung01 TROP2 2L+ NSCLC without actionable mutation
AZD8701# +/- <i>Imfinzi</i> FOXP3 +/- PD-L1 solid tumours	<i>Imfinzi</i> #+adavosertib# PD-L1+Wee1 solid tumours	ceralasertib ATR solid tumours	<i>Imfinzi</i> #+monalizumab# PD-L1+NKG2a solid tumours	<i>Imfinzi</i> #+tremelimumab HIMALAYA PD-L1+CTLA-4 1L HCC	<i>Imfinzi</i> #+/-tremelimumab+CRT ADRIATIC PD-L1+/-CTLA-4+CRT LS-SCLC
MEDI1191 IL12 mRNA solid tumours	IPH5201# CD39 solid tumours	<i>Imfinzi</i> # (platform) COAST PD-L1+multiple novel ONC therapies NSCLC	MEDI5752 PD-1/CTLA-4 solid tumours	<i>Lynparza</i> #+ <i>Imfinzi</i> # DuO-E PARP+PD-L1 1L endometrial cancer	<i>Imfinzi</i> #+tremelimumab+SoC NILE PD-L1+CTLA-4+SoC 1L urothelial cancer
MEDI9253 rNDV IL12 solid tumours	MEDI5752+Lenvatinib PD-1/CTLA-4+VEGF advanced renal cell carcinoma	<i>Imfinzi</i> #+ <i>Lynparza</i> # ORION PD-L1+PARP 1L mNSCLC	Post-1L <i>Tagrisso</i> (platform) ORCHARD EGFR+multiple novel ONC therapies EGFRm NSCLC	monalizumab#+cetuximab (INTERLINK-1) NKG2a+EGFR 2L+ relapsed metastatic HNSCC	<i>Lynparza</i> #+ <i>Imfinzi</i> #+bevacizumab DuO-O PARP+PD-L1+VEGF 1L ovarian
<i>Tagrisso</i> combo# TATTON EGFR+MEK/MET advanced EGFRm NSCLC		<i>Imfinzi</i> +FOLFOX+bevacizumab (platform) COLUMBIA1 PD-L1+chemo+VEGF+multiple novel ONC therapies 1L MSS-CRC	<i>Tagrisso</i> +savolitinib# SAVANNAH EGFR+MET advanced EGFRm NSCLC		savolitinib# + <i>Imfinzi</i> # SAMETA MET+PD-L1 1st-line papillary renal cell carcinoma
		oleclumab+chemo or <i>Imfinzi</i> # + oleclumab + chemo CD73 + chemo or PDL1 + CD73 + chemo pancreatic			

Under review

1 New Molecular Entities

Imfinzi#+/-tremelimumab+chemo
POSEIDON
PD-L1+/-CTLA-4+SoC 1L NSCLC

● Precision medicine approach being explored



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

Q4 2021 Oncology life-cycle management¹ pipeline

Phase 1	Phase 2	Phase 3	Under review
1 Project	7 Projects	32 Projects	3 Projects
<i>Enhertu</i> # (platform) DESTINY-Breast08 ADC breast	<i>Enhertu</i> # (platform) DESTINY-Breast07 ADC breast	<i>Calquence</i> # ECHO BTK inhibitor 1st line MCL	<i>Calquence</i> #+venetoclax+obinutuzumab BTK+BCL-2+anti-CD20 1st line CLL
	<i>Enhertu</i> # DESTINY-Lung01 ADC NSCLC	<i>Enhertu</i> # DESTINY-Breast04 ADC breast	<i>Enhertu</i> # DESTINY-Breast02 ADC breast
	<i>Enhertu</i> # DESTINY-PanTumor01 HER2 targeting ADC HER2-expressing solid tumours	<i>Enhertu</i> # DESTINY-Breast06 ADC breast	<i>Enhertu</i> # DESTINY-Breast09 HER2 targeting ADC HER2+ breast cancer 1L
	<i>Enhertu</i> # DESTINY-PanTumor02 HER2 targeting ADC HER2-expressing solid tumours	<i>Enhertu</i> # DESTINY-Lung04 ADC NSCLC	<i>Enhertu</i> # DESTINY-Gastric04 HER2 targeting ADC HER2+ gastric cancer 2L
	<i>Imfinzi</i> # (platform) BEGONIA PD-L1 1L mTNBC	<i>Imfinzi</i> MERMAID-2 PD-L1 stage II-III premetastatic NSCLC	<i>Imfinzi</i> # CALLA PD-L1 adj. locally-advanced cervical cancer
	<i>Imfinzi</i> # (platform) MAGELLAN PD-L1 1L mNSCLC	<i>Imfinzi</i> # POTOMAC PD-L1 non muscle invasive bladder cancer	<i>Imfinzi</i> # post-SBRT PACIFIC-4 PD-L1 post-SBRT stage I/II NSCLC
	<i>Lynparza</i> # (basket) MK-7339-002 / LYNK002 PARP HRRm cancer	<i>Imfinzi</i> #+CRT PACIFIC-2 PD-L1+CRT NSCLC	<i>Imfinzi</i> #+CRT PACIFIC-5 (China) PD-L1+CRT locally-advanced stage III NSCLC
		<i>Imfinzi</i> #+CTx NIAGARA PD-L1+CTx muscle invasive bladder cancer	<i>Imfinzi</i> #+CTx neoadjuvant AEGEAN PD-L1+CTx locally-advanced stage II-III NSCLC
		<i>Imfinzi</i> #+FLOT MATTERHORN PD-L1+CTx neo-adjuvant/adjuvant gastric cancer	<i>Imfinzi</i> #+VEGF EMERALD-2 PD-L1+VEGF adjuvant HCC
		<i>Lynparza</i> # MONO-OLA1 PARP 1L BRCAwt ovarian cancer	<i>Lynparza</i> # LYNK-003 PARP platinum sensitive 1L colorectal cancer
		<i>Tagrisso</i> LAURA EGFRm locally-advanced unresectable NSCLC	<i>Tagrisso</i> +/-CTx neoadjuvant NeoADAURA EGFR+/-CTx stage II/III resectable EGFRm NSCLC
			<i>Calquence</i> +R-CHOP ESCALADE BTK+R-CHOP 1L DLBCL
			<i>Enhertu</i> # DESTINY-Breast05 ADC breast
			<i>Enhertu</i> # DESTINY-Breast11 ADC breast
			<i>Imfinzi</i> + EV +- treme VOLGA PD-L1 + nectin-4 targeting ADC +/- CTLA4 muscle invasive bladder cancer
			<i>Imfinzi</i> # PEARL PD-L1 1L metastatic NSCLC
			<i>Imfinzi</i> #+CRT KUNLUN PD-L1+CRT locally-advanced esophageal squamous cell carcinoma
			<i>Imfinzi</i> #+CtX MERMAID-1 PD-L1 stage II-III adjuvant NSCLC
			<i>Imfinzi</i> #+CTx TOPAZ-1 PD-L1+CTx 1L biliary tract cancer
			<i>Imfinzi</i> #+VEGF+TACE EMERALD-1 PD-L1+VEGF+TACE locoregional HCC
			<i>Tagrisso</i> +chemo FLAURA2 EGFR+chemo 1L adv EGFRm NSCLC
			<i>Enhertu</i> # DESTINY-Breast03 ADC breast
			<i>Lynparza</i> # OlympiA PARP gBRCA adjuvant breast
			<i>Lynparza</i> +abiraterone# PROpel PARP+NHA prostate cancer

Phase progressions based on first patient dose achievement.

¹ Includes significant life-cycle management projects and parallel indications for assets beyond Phase III

Partnered and/or in collaboration; ¹ Registrational Phase II/III trial

● Precision medicine approach being explored



Q4 2021 BioPharmaceuticals new molecular entity¹ pipeline

Phase 1		Phase 2		Phase 3
14 New Molecular Entities		15 New Molecular Entities		5 New Molecular Entities
AZD2373 Podocyte health nephropathy	AZD2693 nonalcoholic steatohepatitis	AZD1402# inhaled IL-4Ra asthma	AZD4831 MPO HFpEF	brazikumab [¶] IL-23 crohns disease
AZD3366 CD39L3 CV disease	AZD3427 Relaxin ThP CV disease	AZD5718 FLAP coronary artery disease / CKD	AZD7986# DPP1 COPD	epiontersen# LICA hATTR-Polyneuropathy
AZD4041# orexin 1 receptor antagonist opioid use disorder	AZD4604 inhaled JAK1 asthma	AZD8233 hypercholesterolemia cardiovascular	AZD8601# VEGF-A cardiovascular	epiontersen# LICA ATTR-Cardiomyopathy
AZD5055 Porcupine inhibitor IPF	AZD5462 Relaxin mimetic CV disease	AZD9977+ <i>Farxiga</i> MR+SGLT2 HF with CKD	brazikumab IL-23 ulcerative colitis	nirsevimab# RSV mAb-YTE passive RSV immunisation
AZD7503 ASO non-alcoholic steatohepatitis	AZD8630# Inhaled TSLP FAB	cotadutide GLP-1/glucagon T2D / obesity / NASH / DKD	MEDI6570 LOX-1 CV disease	PT027# SABA/ICS asthma
MEDI0618# PAR2 antagonist mAb osteoarthritis pain	MEDI1341# alpha synuclein parkinson's disease	MEDI7352 NGF/TNF OA pain / painful diabetic neuropathy	navafenterol# MABA COPD	
MEDI1814# amyloid β alzheimer's disease	MEDI8367 avb8 chronic kidney disease	tozorakimab (MEDI3506) IL-33 diabetic kidney disease	tozorakimab (MEDI3506) IL-33 AD / COPD / asthma / COVID-19	Under review
			Zibotentan+ <i>Farxiga</i> ZENITH-CKD ETA antagonist+SGLT2 CKD	0 New Molecular 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

● Precision medicine approach being explored



Q4 2021 BioPharmaceuticals life-cycle management¹ pipeline

Phase 1	Phase 2	Phase 3	Under review
0 Projects	5 Projects	14 Projects	1 Project
	<p><i>Fasenra</i> ARROYO IL-5R chronic spontaneous urticaria</p>	<p><i>Breztri</i> LABA/LAMA/ICS asthma</p>	<p><i>Farxiga/Forxiga</i> DAPA-MI SGLT2 prevention of HF and CV death following a myocardial infarction</p>
	<p><i>Fasenra</i> HILLIER IL-5R atopic dermatitis</p>	<p><i>Farxiga/Forxiga</i> DELIVER SGLT2 HFpEF</p>	<p><i>Fasenra</i> FJORD IL-5R bullous pemphigoid</p>
	<p>roxadustat# HIF-PH inhibitor chemo induced anaemia</p>	<p><i>Fasenra</i> MAHALE IL-5R non-cystic fibrosis bronchiectasis</p>	<p><i>Fasenra</i> MANDARA IL-5R EGPA</p>
	<p><i>Saphnelo</i># Type I IFN receptor lupus nephritis</p>	<p><i>Fasenra</i> MESSINA IL-5R eosinophilic esophagitis</p>	<p><i>Fasenra</i> NATRON IL-5R hypereosinophilic syndrome</p>
	<p><i>Tezspire</i> (tezepelumab)# COURSE TSLP COPD</p>	<p><i>Fasenra</i># RESOLUTE IL-5R COPD</p>	<p><i>Lokelma</i> DIALIZE-Outcomes potassium binder CV outcomes in patients on chronic haemodialysis with hyperkaemia</p>
		<p><i>Lokelma</i> STABILIZE-CKD potassium binder hyperkaemia in CKD</p>	<p>roxadustat# HIFPH anaemia MDS</p>
		<p><i>Saphnelo</i># TULIP-SC Type I IFN receptor SLE SC</p>	<p><i>Tezspire</i> (tezepelumab)# WAYPOINT TSLP nasal polyps</p>
			<p><i>Fasenra</i># OSTRO IL-5R nasal polyps</p>

Phase progressions based on first patient dose achievement.

¹ Includes significant life-cycle management projects and parallel indications for assets beyond Phase III

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

● Precision medicine approach being explored



Q4 2021 Rare Disease pipeline¹

Phase 1	Phase 2	Phase 3	Under review
3 Projects	3 Projects	8 Projects	2 Projects
ALXN1720 anti-C5 bi-specific minibody generalised myasthenia gravis	ALXN2050 oral factor D inhibitor paroxysmal nocturnal haemoglobinuria	acoramidis (ALXN2060) oral TTR stabilizer transthyretin amyloid cardiomyopathy (oral ATTR CM)	<i>Ultomiris</i> anti-complement C5 mAb generalised myasthenia gravis (gMG)
ALXN1820 anti-properdin bi-specific haematology	danicopan (ALXN2040) factor D inhibitor geographic atrophy	ALXN1840 bis-choline tetrathiomolybdate Wilson's disease	<i>Ultomiris</i> anti-complement C5 mAb subcutaneous paroxysmal nocturnal haemoglobinuria (PNH) and atypical haemolytic uremic syndrome aHUS)
ALXN1850 next generation asfotase alfa hypophosphatasia	<i>Andexxa</i> anti-factor Xa reversal urgent surgery	CAEL-101 fibrin-reactive mAb AL amyloidosis	
		danicopan (ALXN2040) factor D inhibitor paroxysmal nocturnal haemoglobinuria with extravascular haemolysis	
		<i>Ultomiris</i> anti-complement C5 mAb complement-mediated thrombotic microangiopathy (CMA-TMA)	
		<i>Ultomiris</i> [¶] anti-complement C5 mAb dermatomyositis	
		<i>Ultomiris</i> anti-complement C5 mAb haemtopoietic stem cell transplant-associated thrombotic microangiopathy (HSCT-TMA)	
		<i>Ultomiris</i> anti-complement C5 mAb neuromyelitis optica spectrum disorder (NMOSD)	

Phase progressions based on first patient dose achievement.

¹ Includes new molecular entities and significant life-cycle management projects

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

● Precision medicine approach being explored



Estimated key regulatory submission acceptances

			acoramidis (ALXN2060) Transthyretin Amyloid Cardiomyopathy			
			danicopan (ALXN2040) PNH-EVH			
			Fasenra severe asthma (China)			
	Vaxzevria / COVID-19 Vaccine AstraZeneca (US)		Imfinzi+ tremelimumab + CRT LDS-SCLC ADRIATIC	datopotamab deruxtecan breast cancer TROPION-Breast01		
	PT027 asthma		Imfinzi + tremelimumab + SoC urothelial NILE	capivasertib + fulvestrant + palbociclib 1L locally advanced or mBC CAPitello-292	Orpathys + Imfinzi 1L PRCC SAMETA	
	nirsevimab passive RSV immunisation	ALXN1840 Wilson Disease	datopotamab deruxtecan NSCLC TROPION-Lung01	capivasertib + abiraterone PTEN deficient mHSPC CAPitello-281	monalizumab + cetuximab 2L+ relapsed mHNSCC INTERLINK-1	
NME	Lynparza + abiraterone prostate PROPEL (US & Japan)	eplontersen hATTR-PN	capivasertib + fulvestrant 2L locally advanced or mBC CAPitello-291	camizestrant + palbociclib breast cancer SERENA-4	Lynparza+Imfinzi+ bevacizumab ovarian DUO-O	
	Imfinzi + tremelimumab HCC HIMALAYA	Koselugo NF1 (China) SPRINT	capivasertib + CTx 1L mTNBC CAPitello-290	camizestrant + CDK4/6i 1L HR+ HER2- ESR1m breast cancer SERENA-6	Lynparza + Imfinzi endometrial cancer DUO-E	
	H1 2022	H2 2022	2023	2023+		
LCM	Enhertu DESTINY-Breast04	Calquence 1L CLL ELEVATE-TN (Japan)	Calquence 1L MCL ECHO	Calquence + R-CHOP 1L DLBCL ESCALADE	Imfinzi + EV +- treme VOLGA muscle invasive bladder cancer	Fasenra COPD RESOLUTE
	Imfinzi +CTx biliary tract TOPAZ-1	Enhertu DESTINY-Breast02	Imfinzi + chemo muscle invasive bladder NIAGARA	Calquence + venetoclax + obinutuzumab 1L CLL AMPLIFY	Imfinzi +CTx stage II-III adjuvant NSCLC MERMAID-1	Fasenra nasal polyps ORCHID (China / Japan)
	Xigduo XR/Xigduo type-2 diabetes (China)	Imfinzi + CRT NSCLC PACIFIC-2	Imfinzi + CRT NSCLC PACIFIC-5 (China)	Enhertu DESTINY-Breast05	Imfinzi non muscle invasive bladder POTOMAC	Fasenra non-cystic fibrosis bronchiectasis MAHALE
		Imfinzi + VEGF + TACE locoregional HCC EMERALD-1	Imfinzi + VEGF adjuvant HCC EMERALD-2	Enhertu DESTINY-Breast06	Imfinzi post-SBRT NSCLC PACIFIC-4	Lokelma DIALIZE-Outcomes
		Imfinzi cervical CALLA	Imfinzi neoadjuvant NSCLC AEGEAN	Enhertu DESTINY-Breast09	Imfinzi stage II-III premetastatic NSCLC MERMAID-2	Lokelma STABILIZE-CKD
		Imfinzi NSCLC PEARL	Lynparza platinum sensitive 1L colorectal LYNK-003	Enhertu DESTINY-Breast11	Lynparza 1L BRCAwt ovarian MONO-OLA	roxadustat anemia in MDS
		Farxiga HF (HFpEF) DELIVER	Tagrisso + CTx EGFRm NSCLC FLAURA2	Enhertu DESTINY-Gastric04	Tagrisso stage II/III resectable EGFRm NSCLC NeoADAURA	Saphnelo (anifrolumab) SLE subcutaneous
		Ultomiris Neuromyelitis Optica Spectrum Disorder	Tagrisso locally adv. unresectable NSCLC LAURA	Enhertu DESTINY-Lung04	Breztri/Trixeo asthma KALOS, LOGOS	Tezspire nasal polyposis WAYPOINT
			Fasenra EGPA MANDARA	Imfinzi adjuvant NSCLC BR.31	Farxiga prevention of HF and CV death following a myocardial infarction DAPA-MI	Ultomiris CM-TMA
			Fasenra eosinophilic esophagitis MESSINA	Imfinzi + CRT LA ESCC KUNLUN	Fasenra bullous pemphigoid FIJORD	Ultomiris HSCT-TMA
		Fasenra HES NATRON	Imfinzi + CRT neo-adjuvant/adjuvant gastric MATTERHORN			



Designations in our pipeline

2

Accelerated approvals

Andexxa Acute Major Bleed (US)
Calquence MCL 1L (US)

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Breakthrough / PRIME¹ / Sakigake²

danicopan (ALXN2040) PNH-EVH (US)
danicopan (ALXN2040) PNH-EVH (EU)
Calquence CLL ELEVATE-TN, ASCEND (US)
Calquence MCL 1L (US)
Enhertu HER2+ breast 2L DESTINY-Breast03 (US)
Enhertu HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
Enhertu HER2+/HER2low gastric 3L DESTINY-Gastric01 (JP) ²
Enhertu HER2mut NSCLC 2L+ DESTINY-Lung01 (US)
Forxiga CKD DAPA-CKD (US)
Koselugo NFI type 1 SPRINT (US)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (US)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (CN)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (EU) ¹
Soliris GBS (JP) ²
Tagrisso adjuvant NSCLC ADAURA (US)
Tezspire asthma NAVIGATOR (US)

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Fast Track

Sapnelo SLE (US)
CAEL-101 AL amyloidosis (US)
cotadutide NASH (US)
Fasenra EG/EGE HUDSON (US)
Forxiga CKD DAPA-CKD (US)
Forxiga MI RRCT DAPA-MI (US)
Lokelma ESRD DIALIZE-OUTCOMES (US)
nirsevimab RSV mAb-YTE MELODY-MEDLEY (US)

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Priority Review

Brilinta stroke THALES (US)
Calquence MCL 1L (US)
Enhertu HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
Enhertu HER2+ breast 2L DESTINY-Breast03 (US)
Forxiga CKD DAPA-CKD (US)
Forxiga CKD DAPA-CKD (JP)
Koselugo NFI type 1 SPRINT (US)
Lynparza gBRCA adj breast OlympiA (US)
Roxadustat chronic kidney disease (CN)
Tagrisso adjuvant NSCLC (ADAURA) (US)
Tezspire asthma NAVIGATOR (US)
Ultomiris gMG (US)

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Orphan

ALXN1830 WAIHA (EU)
danicopan (ALXN2040) PNH (US)
danicopan (ALXN2040) PNH (EU)
ALXN2050 PNH (US)
ALXN2050 PNH (EU)
ALXN1840 WD (US)
ALXN1840 WD (EU)
Andexxa Acute Major Bleed (JP)
CAEL-101 AL amyloidosis (US)
CAEL-101 AL amyloidosis (EU)
Calquence CLL 1L (US)
Calquence CLL 1L (EU)
Calquence MCL 1L (US)
Enhertu HER2+/HER2low gastric 3L DESTINY-Gastric01 (US)
eplontersen transthyretin-mediated amyloidosis (US)
Fasenra EG/EGE HUDSON (US)
Fasenra EGPA MANDARA (US)
Fasenra EOE MESSINA (US)
Fasenra HES NATRON (US)
Imfinzi + CTx Biliary Tract 1L TOPAZ-1 (US)
Imfinzi +/- treme HCC 1L (EU)
Imfinzi +/- treme HCC 1L (US)
Koselugo NFI type 1 SPRINT (US)
Koselugo NFI type 1 SPRINT (EU)
Koselugo NFI type 1 SPRINT (JP)
Lynparza gBRCA adj breast OlympiA (JP)
Tezspire Eosinophilic Esophagitis (US)
Ultomiris DM (US)
Ultomiris HSCT-TMA (US)
Ultomiris SC PNH (US)

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

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

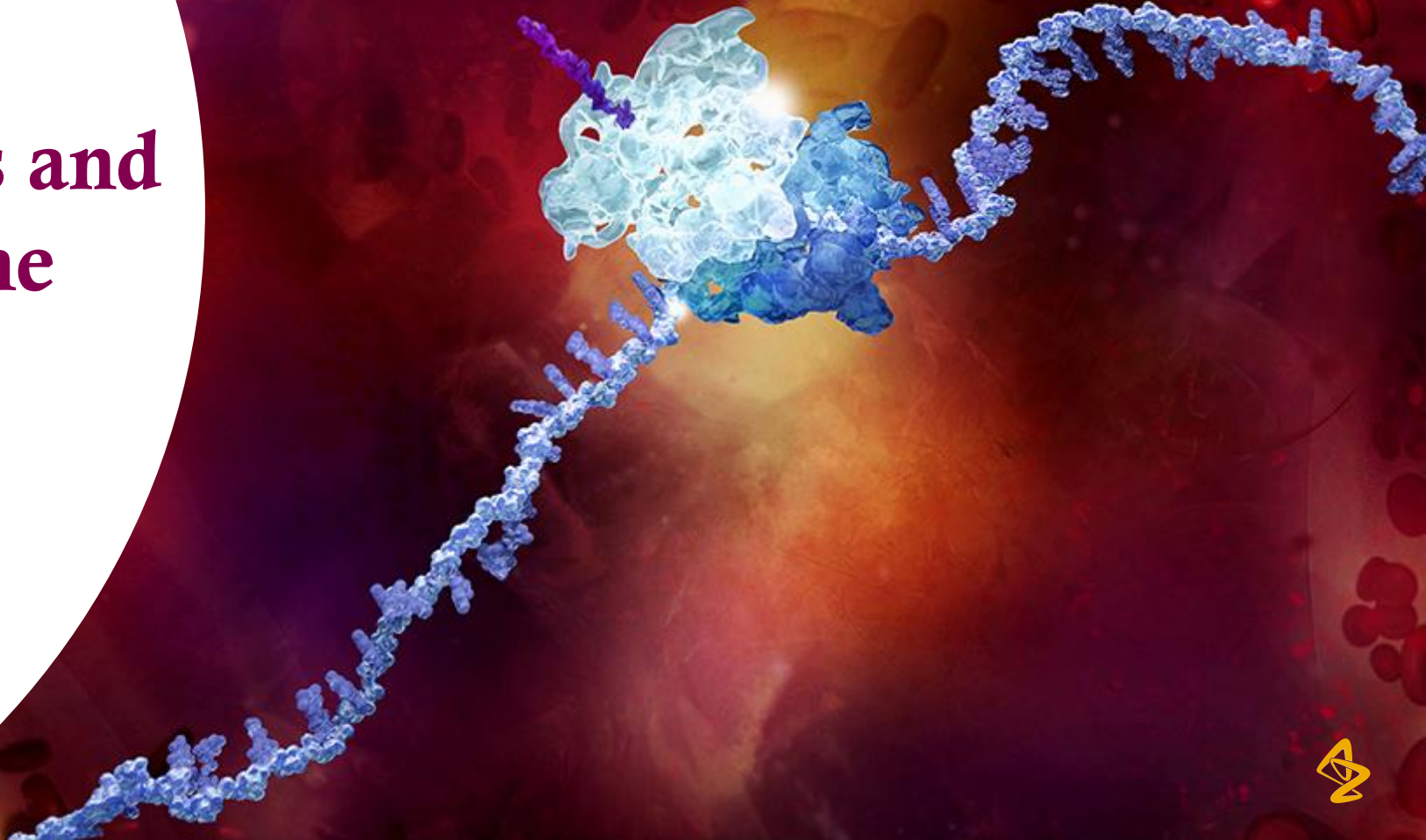
FAST TRACK is a process designed to facilitate the development, and expedite the review of medicines to treat serious conditions and fill an unmet medical need.

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

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



**Oncology –
approved medicines and
late-stage pipeline**



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ADAURA NCT02511106	Adjuvant EGFRm NSCLC	682	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> QD following complete tumour resection, with or without chemo Arm 2: placebo Global trial - 25 countries	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS Rate, OS, OS Rate, QoL 	<ul style="list-style-type: none"> 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	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> Arm 2: placebo Global trial - 17 countries	<ul style="list-style-type: none"> Primary endpoint: PFS (BICR) Secondary endpoints: CNS PFS, OS, DoR, ORR, DCR 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: 2023
Phase II ELIOS NCT03239340	EGFR TKI treatment-naïve patients with locally-advanced or metastatic EGFRm NSCLC	150	<ul style="list-style-type: none"> Single arm trial - <i>Tagrisso</i> Global trial - five countries	<ul style="list-style-type: none"> 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 endpoints: PFS, ORR, DoR 	<ul style="list-style-type: none"> FPCD: Q2 2018
Phase I ODIN-BM NCT03463525	Patients with EGFRm NSCLC with brain metastases	8	<ul style="list-style-type: none"> Single-arm trial - <i>Tagrisso</i> 	<ul style="list-style-type: none"> Primary endpoints: assessments of brain standard uptake value (SUV) and pharmacokinetics (PK) Secondary endpoint: PK 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2020 Data readout: Q4 2021

Oncology
CVRM
R&I
Other
COVID-19
Rare Disease



Tagrisso (highly-selective, irreversible EGFRi)

NSCLC, combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III NeoADAURA NCT04351555	Neoadjuvant EGFRm NSCLC	351	<ul style="list-style-type: none"> Arm 1: placebo plus pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: <i>Tagrisso</i> plus pemetrexed/carboplatin or pemetrexed/cisplatin Arm 3: <i>Tagrisso</i> Global trial – 23 countries	<ul style="list-style-type: none"> Primary endpoint: mPR Secondary endpoints: cPR, EFS, DFS, OS 	<ul style="list-style-type: none"> FPCD Q1 2021 Data anticipated: 2023+
Phase III FLAURA2 NCT04035486	1st-line EGFRm NSCLC	586	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> plus pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: <i>Tagrisso</i> Global trial – 23 countries	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, LOS, ORR, DoR, Depth of response, PFS2, QoL, PK 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: 2023
Phase III COMPEL NCT04765059	EGFRm metastatic NSCLC patients who have progressed extracranially following 1L treatment with <i>Tagrisso</i>	204	<ul style="list-style-type: none"> Arm 1: <i>Tagrisso</i> plus pemetrexed/carboplatin or pemetrexed/cisplatin Arm 2: placebo plus pemetrexed/carboplatin or pemetrexed/cisplatin Global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: Intracranial PFS, Extracranial PFS, OS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+
Phase II SAVANNAH NCT03778229	EGFRm / MET+, locally advanced or metastatic NSCLC who have progressed following treatment with <i>Tagrisso</i>	259	<ul style="list-style-type: none"> Single arm trial: <i>Tagrisso</i> + <i>Orpathys</i> Global trial	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints include PFS, DoR and OS 	<ul style="list-style-type: none"> FPCD Q1 2019 Data anticipated: H2 2022
Phase II ORCHARD NCT03944772	Advanced EGFRm NSCLC patients who have progressed on first line <i>Tagrisso</i> treatment	182	Modular design platform trial: <ul style="list-style-type: none"> Module 1: <i>Tagrisso</i> + <i>Orpathys</i> (cMET) Module 2: <i>Tagrisso</i> + gefitinib (EGFRm) Module 3: <i>Tagrisso</i> + necitumumab (EGFRm) Module 4: carboplatin + pemetrexed + <i>Imfinzi</i> Module 5: <i>Tagrisso</i> + alectinib (ALK) Module 6: <i>Tagrisso</i> + selpercatinib (RET fusion) Module 7: <i>Imfinzi</i> + etoposide + carboplatin or cisplatin Module 8: <i>Tagrisso</i> + pemetrexed + carboplatin or cisplatin Module 9: <i>Tagrisso</i> + <i>Koselugo</i> No intervention: observational cohort Global trial - 8 countries	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, DoR, OS, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: 2023+
Phase II NCT04606771	EGFRm/MET amplified advanced NSCLC	56	<ul style="list-style-type: none"> <i>Tagrisso</i> and <i>Orpathys</i> contribution of components 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, DoR, OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022

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	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + SoC chemo Arm 2: placebo + SoC chemo 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS, OS 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: 2023+
Phase III MERMAID-2 NCT04642469	Completely resected Stage II-III NSCLC	284	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoints: DFS, PFS, OS 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+
Phase III AEGEAN NCT03800134	Neoadjuvant NSCLC patients Stage II and III resected NSCLC (incl. EGFR/ALK positive)	800	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + platinum-based chemo Arm 2: placebo + platinum-based chemo 	<ul style="list-style-type: none"> Primary endpoints: pCR, EFS Secondary endpoint: mPR 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: 2023
Phase III ADJUVANT BR.31 NCT02273375 Partnered	Adjuvant NSCLC patients Stage Ib (≥4cm) – stage IIIa resected (incl. EGFR/ALK positive)	1360	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> mg/kg i.v. Q4W x 12m Arm 2: placebo Global trial	<ul style="list-style-type: none"> Primary endpoint: DFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2015 LPCD: Q1 2020 Data anticipated: 2023+
Phase III PACIFIC-2 NCT03519971	Unresected, locally-advanced NSCLC	300	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W + chemo/RT Arm 2: placebo + chemo/RT ex US global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q3 2019 Data anticipated: H1 2022
Phase III PACIFIC-4 NCT03833154	<i>Imfinzi</i> with SBRT in unresected, Stage I/II NSCLC	630	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W with definitive SBRT Arm 2: placebo with definitive SBRT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data anticipated: 2023
Phase III PACIFIC-5 NCT03706690	Unresected, locally-advanced NSCLC	360	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> i.v. Q4W following chemo/RT Arm 2: placebo following chemo/RT ex US global trial, China focus	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: 2023
Phase III PACIFIC-8 NCT05211895	Unresected, locally-advanced NSCLC	860	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + domvanalimab following chemo/RT Arm 2: <i>Imfinzi</i> + PBO following chemo/RT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: 2023+



Imfinzi (PD-L1 mAb)

Lung cancer, early disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II HUDSON NCT03334617	NSCLC, patients who progressed on an anti-PD-1/PD-L1 containing therapy	340	Open-label, biomarker-directed, multicentre trial <ul style="list-style-type: none"> Module 1: <i>Imfinzi</i> and <i>Lynparza</i> Module 2: <i>Imfinzi</i> and danvatirsen Module 3: <i>Imfinzi</i> and ceralasertib (AZD6738) Module 4: <i>Imfinzi</i> and vistusertib Module 5: <i>Imfinzi</i> and oleclumab Module 6: <i>Imfinzi</i> and <i>Enhertu</i> Module 7: <i>Imfinzi</i> and cediranib Module 8: ceralasertib Module 9: <i>Imfinzi</i> and ceralasertib 	<ul style="list-style-type: none"> Primary outcome: ORR Secondary outcomes: efficacy including OS, PFS, DCR, and safety and tolerability, DoR 	<ul style="list-style-type: none"> FPCD: Q1 2018 Data anticipated: 2023+
Phase II COAST NCT03822351	Stage III NSCLC unresectable	189	<ul style="list-style-type: none"> Arm A: <i>Imfinzi</i> Arm B: <i>Imfinzi</i> + oleclumab Arm C: <i>Imfinzi</i> + monalizumab 	<ul style="list-style-type: none"> Primary endpoint: OR per RECIST v1.1 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data readout: Q3 2021
Phase II NeoCOAST NCT03794544	Resectable, early-stage NSCLC	84	<ul style="list-style-type: none"> Arm A: <i>Imfinzi</i> Arm B: <i>Imfinzi</i> + oleclumab Arm C: <i>Imfinzi</i> + monalizumab Arm D: <i>Imfinzi</i> + danvatirsen 	<ul style="list-style-type: none"> Primary endpoint: Major pathological response rate 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: H1 2022
Phase I/II SCoPe-D1 NCT04870112	NSCLC SCLC	124	Open-label, multicentre trial to evaluate the safety, pharmacokinetics, and preliminary efficacy of subcutaneous <i>Imfinzi</i>	<ul style="list-style-type: none"> Primary endpoints: PK, safety 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+



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	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> Q4W Arm 2: chemotherapy Asia trial	<ul style="list-style-type: none"> Primary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q1 2017 LPCD: Q1 2019 Data anticipated: H1 2022
Phase III POSEIDON NCT03164616	NSCLC 1L	1000	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + chemo Arm 2: <i>Imfinzi</i> + tremelimumab + chemo Arm 3: SoC 	<ul style="list-style-type: none"> Primary endpoints: OS, PFS 	<ul style="list-style-type: none"> FPCD: Q2 2017 LPCD: Q4 2018 Data readout: Q4 2019 PFS primary endpoint met OS data readout: Q2 2021
Phase II MAGELLAN NCT03819465	NSCLC 1L	212	<ul style="list-style-type: none"> Arm A1: <i>Imfinzi</i> Arm A2: <i>Imfinzi</i> + danvatirsen Arm A3: <i>Imfinzi</i> + oleclumab Arm A3: MEDI5752 Arm B1: <i>Imfinzi</i> + Investigator's choice of chemo Arm B2: <i>Imfinzi</i> + danvatirsen + Investigator's choice of chemo Arm B3: <i>Imfinzi</i> + oleclumab + Investigator's choice of chemo Arm B4: MEDI5752 	<ul style="list-style-type: none"> Primary endpoints: safety & tolerability Secondary endpoints: ORR, DoR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: 2023+
Phase Ib Study 006 NCT02000947	NSCLC (IO naïve and IO pretreated patient cohorts)	459	Dose escalation: <ul style="list-style-type: none"> Minimum 5 cohorts exploring various treme Q4W and <i>Imfinzi</i> i.v. Q4W dose combinations, higher dose levels and alternate Q2 schedule added with amendment Dose expansion: <ul style="list-style-type: none"> MTD for the combination in escalation to be explored in expansion North American, EU and ROW trial centres 	<ul style="list-style-type: none"> Primary endpoints: safety, Optimal biologic dose for the combination, OR Secondary endpoints include: antitumour activity, PK and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q4 2013 LPCD: Q4 2016 Data readout: Q3 2020



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

SCLC, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III ADRIATIC NCT03703297	Limited stage SCLC 1L following platinum-based concurrent chemoradiation therapy	600	<ul style="list-style-type: none">Arm 1: <i>Imfinzi</i> + tremelimumab (4 doses)Arm 2: <i>Imfinzi</i>Arm 3: placebo	<ul style="list-style-type: none">Primary endpoints: PFS, OS	<ul style="list-style-type: none">FPCD: Q4 2018Data anticipated: H2 2022



Imfinzi (PD-L1 mAb)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III POTOMAC NCT03528694	Non-muscle invasive bladder cancer	1018	<ul style="list-style-type: none"> Arm 1: BCG (Induction + maintenance) Arm 2: <i>Imfinzi</i> + BCG (Induction only) Arm 3: <i>Imfinzi</i> + BCG (Induction + maintenance) 	<ul style="list-style-type: none"> Primary endpoint: DFS 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q4 2020 Data anticipated: 2023+
Phase III NIAGARA NCT03732677	Muscle-invasive bladder cancer	1064	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> in combination with gemcitabine + cisplatin, <i>Imfinzi</i> maintenance Arm 2: gemcitabine + cisplatin 	<ul style="list-style-type: none"> Copriary endpoints: pCR , EFS 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q3 2021 Data anticipated: 2023
Phase III EMERALD-1 NCT03778957	Locoregional HCC	710	<ul style="list-style-type: none"> Arm 1: TACE in combination with <i>Imfinzi</i> Arm 2: TACE in combination with <i>Imfinzi</i> + bevacizumab Arm 3: TACE in combination with placebo 	<ul style="list-style-type: none"> Primary endpoint: PFS for Arm 1 vs Arm 3 Secondary endpoint: PFS for Arm 2 vs Arm 3 , OS 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q3 2021 Data anticipated: H2 2022
Phase III EMERALD-2 NCT03847428	Adjuvant therapy in HCC	888	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + bevacizumab Arm 2: <i>Imfinzi</i> + placebo Arm 3: placebo + placebo 	<ul style="list-style-type: none"> Primary endpoint: PFS for Arm 1 vs Arm 2 Secondary endpoints: PFS Arm 2 vs Arm 3, OS, RFS at 24m 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data anticipated: 2023
Phase III KUNLUN NCT04550260	Locally advanced, unresectable ESCC	600	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + definitive CRT Arm 2: placebo + definitive CRT 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023+
Phase III MATTERHORN NCT04592913	Resectable GC/GEJC	900	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + FLOT Arm 2: placebo + FLOT 	<ul style="list-style-type: none"> Primary endpoint: EFS Secondary endpoints: OS Arm 1 vs Arm 2, pCR Arm 1 vs Arm 2 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023+
Phase III SAMETA NCT05043090	MET-Driven, unresectable and locally advanced or metastatic papillary renal cell carcinoma	200	<ul style="list-style-type: none"> <i>Orpathys</i> + <i>Imfinzi</i> versus sunitinib and <i>Imfinzi</i> monotherapy 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints include OS, ORR, DoR and DCR 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+
Phase Ib/II COLUMBIA 1 NCT04068610	1L metastatic MSS-CRC	112	<ul style="list-style-type: none"> Part 1 S1 FOLFOX + bevacizumab + <i>Imfinzi</i> + oleclumab Part 2 Control 1 FOLFOX + bevacizumab Part 2 E1 FOLFOX + bevacizumab + <i>Imfinzi</i> + oleclumab 	<ul style="list-style-type: none"> Primary endpoints: <ul style="list-style-type: none"> Part 1: safety Part 2: efficacy (OR) Secondary endpoints: <ul style="list-style-type: none"> Part 1: efficacy (OR), BOR, DoR, PFS Part 2: safety and efficacy (BOR, DoR, DC, PFS, OS) 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: 2023+



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	1220.	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + tremelimumab + SoC Arm 2: <i>Imfinzi</i> + SoC Arm 3: SoC 	<ul style="list-style-type: none"> Primary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q2 2021 Data anticipated: 2023
Phase III HIMALAYA NCT03298451	HCC 1L	1324	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + tremelimumab Arm 2: <i>Imfinzi</i> Arm 3: sorafenib 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: PFS, TTP, ORR 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2019 Data readout: Q4 2021 Primary endpoint met
Phase III TOPAZ-1 NCT03875235	BTC 1L	810	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + gemcitabine + cisplatin Arm 2: placebo + gemcitabine + cisplatin Global trial	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: PFS, ORR, DoR 	<ul style="list-style-type: none"> FPCD Q2 2019 LPCD: Q4 2020 Data readout: Q4 2021 Primary endpoint met
Phase III CALLA NCT03830866	Locally advanced cervical cancer	770	<ul style="list-style-type: none"> Arm 1 <i>Imfinzi</i> + EBRT + brachytherapy with platinum Arm 2 placebo + EBRT + brachytherapy with platinum Global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, CR rate, DoR, ORR, safety/tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q4 2020 Data anticipated: H1 2022
Phase III VOLGA NCT04960709	Muscle invasive bladder cancer ineligible to cisplatin	830	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + tremelimumab + enfortumab vedotin Arm 2: <i>Imfinzi</i> + enfortumab vedotin Arm 3: SoC cystectomy 	<ul style="list-style-type: none"> Primary endpoints: safety, EFS, pCR Secondary endpoints: OS 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+



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

Other cancers, advanced disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II BEGONIA NCT03742102	mTNBC 1L	220	<ul style="list-style-type: none"> Arm 1 <i>Imfinzi</i> + paclitaxel Arm 2 <i>Imfinzi</i> + paclitaxel + capivasertib Arm 5 <i>Imfinzi</i> + paclitaxel + oleclumab Arm 6 <i>Imfinzi</i> + <i>Enhertu</i> Arm 7 <i>Imfinzi</i> + datopotamab deruxtecan Global trial	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: ORR, PFS, DoR, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: 2023+
Phase I/II Study 1108 NCT01693562	Solid tumours	1022	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	<ul style="list-style-type: none"> Primary endpoints: safety, optimal biologic dose Secondary endpoints include: PK, immunogenicity and antitumour activity 	<ul style="list-style-type: none"> FPCD: Q3 2012 LPCD: Q4 2016 Data readout: Q2 2020
Phase I CLOVER NCT03509012	HNSCC, NSCLC, SCLC	167	<i>Imfinzi</i> +/- treme in combination with chemoradiation in advanced solid tumours	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q2 2018 Dara readout: Q4 2021



Lynparza (PARP inhibitor)

Multiple cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III OlympiA NCT02032823 Partnered	BRCAm adjuvant breast cancer	1836	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> BID 12-month duration Arm 2: placebo 12-month duration Global trial partnership with Breast International Group and National Cancer Institute/NRG Oncology	<ul style="list-style-type: none"> Primary endpoint: invasive disease-free survival (iDFS) Secondary endpoint: distant disease-free survival and OS 	<ul style="list-style-type: none"> FPCD: Q2 2014 LPCD: Q2 2019 Data readout: Q1 2021 Primary endpoint met
Phase III MONO-OLA1 NCT04884360	BRCAwT advanced ovarian cancer 1L maintenance	420	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> Arm 2: placebo Global trial, 12 countries	<ul style="list-style-type: none"> Primary endpoints: PFS (BRCAwT HRD+ve), PFS (BRCAwT) Secondary endpoints: OS, TFST, PFS2 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+



Lynparza (PARP inhibitor)

Other combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III PROpel NCT03732820	Metastatic castration-resistant prostate cancer 1L	904	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> + abiraterone Arm 2: placebo + abiraterone Global trial, including China cohort	<ul style="list-style-type: none"> Primary endpoint: rPFS Secondary endpoints: TFST, TTPP, OS 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data readout: Q3 2021 Primary endpoint met
Phase III LYNK-003 NCT04456699 Partnered	Advanced colorectal cancer 1L maintenance	525	<ul style="list-style-type: none"> Arm 1: bevacizumab + 5-FU maintenance Arm 2: bevacizumab + <i>Lynparza</i> maintenance Arm 3: <i>Lynparza</i> maintenance Global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, AEs 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: 2023
Phase II/III COCOS NCT02502266 Externally sponsored	Recurrent platinum resistant/refractory ovarian cancer	680	<ul style="list-style-type: none"> Arm 1: chemo Arm 2: cediranib + <i>Lynparza</i> Arm 3: cediranib Arm 4: <i>Lynparza</i> US, Canada	<ul style="list-style-type: none"> Primary endpoints: PFS, OS Secondary endpoints: ORR, QoL, safety 	<ul style="list-style-type: none"> FPCD: Q2 2016 Data anticipated: 2023
Phase II LYNK-002 NCT03742895 Partnered	HRRm or HRD-positive advanced cancer	390	<ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> Global trial	<ul style="list-style-type: none"> Primary endpoints: ORR Secondary endpoints: DOR, OS, PFS, AE, Prog by CA-125 	<ul style="list-style-type: none"> FPCD: Q1 2019



Lynparza (PARP inhibitor)

Imfinzi combinations

Trial	Population	Patients	Design	Endpoints	Status
Phase III DuO-O NCT03737643	Advanced ovarian cancer 1L	1,256	Non tBRCAm (tumour BRCA) patients <ul style="list-style-type: none"> Arm 1: bevacizumab Arm 2: bevacizumab + <i>Imfinzi</i> Arm 3: bevacizumab + <i>Imfinzi</i> + <i>Lynparza</i> tBRCAm patients <ul style="list-style-type: none"> bevacizumab (optional) + <i>Imfinzi</i> + <i>Lynparza</i> Global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, PFS2 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: 2023
Phase III DuO-E NCT04269200	Advanced and recurrent endometrial cancer 1L	699	<ul style="list-style-type: none"> Arm 1: chemo + <i>Imfinzi</i> placebo followed by <i>Imfinzi</i> placebo and <i>Lynparza</i> placebo Arm 2: chemo + <i>Imfinzi</i> followed by <i>Imfinzi</i> + <i>Lynparza</i> placebo Arm 3: chemo + <i>Imfinzi</i> followed by <i>Imfinzi</i> + <i>Lynparza</i> Global Trial	<ul style="list-style-type: none"> Primary endpoint PFS Secondary endpoints: OS, PFS2, ORR, DoR 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: 2023
Phase II ORION NCT03775486	Stage IV NSCLC whose disease has not progressed following SoC chemo + <i>Imfinzi</i> maintenance therapy 1L	250	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Lynparza</i> Arm 2: <i>Imfinzi</i> + placebo Global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, PFS in HRRm, PK, ADA 	<ul style="list-style-type: none"> FPCD Q1 2019 Data readout: Q2 2021
Phase II BAYOU NCT03459846	Platinum-ineligible unresectable Stage IV urothelial cancer	154	<ul style="list-style-type: none"> Arm 1: <i>Imfinzi</i> + <i>Lynparza</i> Arm 2: <i>Imfinzi</i> + placebo Global trial	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, DoR, ORR, PFS in HRRm, PFS6, PK, ADA, PRO 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q3 2019 Data readout: Q2 2021
Phase I / II MEDIOLA NCT02734004	gBRCAm ovarian cancer 2L+ gBRCAm HER2-negative breast cancer 1-3L SCLC 2L+ Gastric cancer 2L+	148	Dose until progression <ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> + <i>Imfinzi</i> Global trial	<ul style="list-style-type: none"> Primary endpoints: DCR at 12 weeks, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2016 LPCD: Q2 2017
Phase I / II MEDIOLA (Ovarian expansion) NCT02734004	gBRCAm ovarian cancer 2L+ Non-gBRCAm ovarian cancer 2L+ Non-gBRCAm ovarian cancer 2L+	115	Dose until progression <ul style="list-style-type: none"> Arm 1: <i>Lynparza</i> + <i>Imfinzi</i> Arm 2: <i>Lynparza</i> + <i>Imfinzi</i> Arm 3: <i>Lynparza</i> + <i>Imfinzi</i> + bevacizumab Global trial	<ul style="list-style-type: none"> Primary endpoints: DCR at 12 weeks, ORR, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q2 2020



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
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 <ul style="list-style-type: none"> • <i>Enhertu</i> • Physician's choice of lapatinib + capecitabine or trastuzumab + capecitabine 	<ul style="list-style-type: none"> • Primacy endpoint: PFS • Secondary endpoints: OS, ORR, DoR, CBR 	<ul style="list-style-type: none"> • FPCD: Q3 2018 • LPCD: Q4 2020 • Data anticipated: H2 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 <ul style="list-style-type: none"> • <i>Enhertu</i> • Ado-trastuzumab emtansine 	<ul style="list-style-type: none"> • Primary endpoint: PFS • Secondary endpoints: OS, ORR, DoR, CBR 	<ul style="list-style-type: none"> • FPCD: Q3 2018 • LPCD: Q2 2020 • Data readout: Q3 2021 • Primary endpoint met
Phase III DESTINY-Breast04 NCT03734029	HER2-low, unresectable and/or metastatic breast cancer patients	540	Randomised open label parallel assignment <ul style="list-style-type: none"> • <i>Enhertu</i> • Physician's choice of SoC chemo (choice of capecitabine, eribulin, gemcitabine, paclitaxel or nab-paclitaxel) 	<ul style="list-style-type: none"> • Primary end point: PFS • Secondary endpoints: OS, DoR, ORR 	<ul style="list-style-type: none"> • FPCD: Q4 2018 • LPCD: Q4 2020 • Data anticipated: H1 2022
Phase III DESTINY-Breast05 NCT04622319	High-risk HER2-positive patients with residual invasive breast cancer following neoadjuvant therapy	1600	Randomised open label parallel assignment <ul style="list-style-type: none"> • <i>Enhertu</i> • Ado-trastuzumab emtansine 	<ul style="list-style-type: none"> • Primary endpoint: IDFS • Secondary endpoints: DFS, OS, DRFI, BMFI 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • Data anticipated: 2023+
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 <ul style="list-style-type: none"> • <i>Enhertu</i> • Investigator's choice standard of care chemotherapy (capecitabine, paclitaxel, nab-paclitaxel) 	<ul style="list-style-type: none"> • Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR 	<ul style="list-style-type: none"> • FPCD: Q3 2020 • Data anticipated: 2023
Phase III DESTINY-Breast09 NCT04784715	HER2-positive, metastatic breast cancer, no prior therapy for advanced or metastatic disease	1134	Randomised, parallel assignment <ul style="list-style-type: none"> • <i>Enhertu</i> + placebo • <i>Enhertu</i> + pertuzumab • Standard of care 	<ul style="list-style-type: none"> • Primary endpoint: PFS • Secondary endpoints: OS, DoR, ORR 	<ul style="list-style-type: none"> • FPCD: Q2 2021 • Data anticipated: 2023+
Phase III DESTINY-Breast11 NCT05113251	High-risk HER2-positive early non-metastatic breast cancer	624	Randomized open label parallel assignment <ul style="list-style-type: none"> • <i>Enhertu</i> • <i>Enhertu</i>, followed by THP • doxorubicin and cyclophosphamide, followed by THP 	<ul style="list-style-type: none"> • Primary endpoint: pCR • Secondary endpoints: EFS, IDFS, OS 	<ul style="list-style-type: none"> • FPCD: Q4 2021 • Data anticipated: 2023+



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II DESTINY-Breast07 NCT04538742	HER2-positive metastatic breast cancer	450	Randomised open label sequential assignment <ul style="list-style-type: none"> • <i>Enhertu</i> • <i>Enhertu</i> + <i>Imfinzi</i> • <i>Enhertu</i> + pertuzumab • <i>Enhertu</i> + paclitaxel • <i>Enhertu</i> + <i>Imfinzi</i> + paclitaxel • <i>Enhertu</i> + tucatinib 	<ul style="list-style-type: none"> • Primary endpoints: AE, SAE • Secondary endpoints: ORR, PFS, DoR, OS 	<ul style="list-style-type: none"> • FPCD: Q1 2021 • Data anticipated: 2023+
Phase Ib DESTINY-Breast08 NCT04556773	HER2-low metastatic breast cancer	185	Non-randomised open label parallel assignment <ul style="list-style-type: none"> • <i>Enhertu</i> + capecitabine • <i>Enhertu</i> + <i>Imfinzi</i> + paclitaxel • <i>Enhertu</i> + capivasertib • <i>Enhertu</i> + anastrozole • <i>Enhertu</i> + <i>Faslodex</i> 	<ul style="list-style-type: none"> • Primary endpoints: AE, SAE • Secondary endpoints: ORR, PFS, DoR, OS 	<ul style="list-style-type: none"> • FPCD: Q1 2021 • Data anticipated: 2023+



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Gastric cancer

Trial	Population	Patients	Design	Endpoints	Status
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 <ul style="list-style-type: none"> Enhertu SoC chemo 	<ul style="list-style-type: none"> Primary endpoint: OS Secondary endpoints: ORR, DoR, PFS, DcR, safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: 2023+
Phase II DESTINY-Gastric01 NCT03329690	HER2-overexpressing advanced gastric or gastroesophageal junction adenocarcinoma patients who have progressed on two prior treatment regimens	233	Randomised open label parallel assignment <ul style="list-style-type: none"> Enhertu SoC chemo Two additional open label patient cohorts with lower levels of HER2 expression Japan and Korea 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, OS, DoR, DCR, TTF, range of PK endpoints 	<ul style="list-style-type: none"> 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, in patients who have progressed on or after trastuzumab containing regimen	79	Open label single group assignment <ul style="list-style-type: none"> Enhertu Western population	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, ORR, OS, DoR 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q4 2020 Data readout: Q2 2021
Phase Ib/II DESTINY-Gastric03 NCT04379596	HER2-overexpressing gastric or gastroesophageal junction cancer patients	255	Open label parallel assignment Part 1: To determine recommended Phase II combination dose <ul style="list-style-type: none"> 5 Arms combine <i>Enhertu</i> with standard of care chemotherapies (5-FU, capecitabine, oxaliplatin) and / or durvalumab Part 2: To assess efficacy of the selected combinations <ul style="list-style-type: none"> Arm 2A: Standard chemotherapy (control) Arm 2B: <i>Enhertu</i> monotherapy Arm 2C: <i>Enhertu</i> with chemotherapy Arm 2D: <i>Enhertu</i> with chemotherapy and pembrolizumab Arm 2E: <i>Enhertu</i> and pembrolizumab 	<ul style="list-style-type: none"> Part 1 Primary endpoint: safety Part 2 Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS, range of PK endpoints, ADAs 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: 2023
Phase II DESTINY-Gastric06 NCT04989816	HER2-positive gastric cancer or gastro-esophageal junction adenocarcinoma patients who have progressed on two prior treatment regimens	75	Open label single group assignment <ul style="list-style-type: none"> Enhertu China	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: PFS, ORR, DCR, OS, DoR, safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: 2023



Enhertu (trastuzumab deruxtecan, HER2 ADC)

Other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III DESTINY-Lung04 NCT05048797	HER2 mutated, unresectable, locally advanced/metastatic NSCLC	264	Randomised parallel group assignment <ul style="list-style-type: none"> Arm 1: <i>Enhertu</i> Arm 2: SoC Treatment (platinum, pemetrexed and pembrolizumab) 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: OS, ORR, DoR, safety 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+
Phase II DESTINY-Lung01 NCT03505710	HER2-over-expressing or mutated, unresectable and/or metastatic NSCLC	170	Non randomised parallel group assignment <ul style="list-style-type: none"> <i>Enhertu</i> 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, PFS, OS 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data readout: Q3 2021 Positive primary results
Phase II DESTINY-Lung02 NCT04644237	HER2-mutated, unresectable and/or metastatic NSCLC	150	Randomised parallel group assignment <ul style="list-style-type: none"> Arm 1: <i>Enhertu</i> 6.4 mg/kg Arm 2: <i>Enhertu</i> 5.4mg/kg 	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS, PK 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated 2023
Phase Ib DESTINY-Lung03 NCT04686305	HER2-over-expressing, unresectable and/or metastatic NSCLC	136	Non randomised parallel group assignment Part 1: To determine recommended combination dose <ul style="list-style-type: none"> 3 Arms combine <i>Enhertu</i> with standard of care chemotherapies (cisplatin, carboplatin or pemetrexed) and <i>Imfinzi</i>. Arm 1D: <i>Enhertu monotherapy arm</i> Part 2: To assess efficacy of the selected combinations <ul style="list-style-type: none"> Arm 1: <i>Enhertu</i> + cisplatin + <i>Imfinzi</i> Arm 2: <i>Enhertu</i> + carboplatin + <i>Imfinzi</i> Arm 3: <i>Enhertu</i> + pemetrexed + <i>Imfinzi</i> Arm 4: <i>Enhertu</i> + <i>Imfinzi</i> 	<ul style="list-style-type: none"> Primary endpoint: safety Secondary endpoints: ORR, DoR, DCR, PFS, OS, range of PK endpoints 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+



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 • <i>Enhertu</i>	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, OS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023
Phase II DESTINY-PanTumour01 NCT04639219	HER2 mutant tumours	100	Non-randomised single group assignment • <i>Enhertu</i>	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, DCR, PFS, PK 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023
Phase II DESTINY-CRC02 NCT04744831	HER2 overexpressing advanced or metastatic colorectal cancer	120	Randomised parallel group assignment • Arm 1: <i>Enhertu</i> 6.4 mg/kg • Arm 2: <i>Enhertu</i> 5.4mg/kg	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoint: PFS, OS, DoR, DCR, range of PK endpoints 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023
Phase Ib U106 NCT04042701	HER2 expressing locally advanced/metastatic breast or NSCLC	115	Non randomised parallel group assignment • <i>Enhertu</i> + pembrolizumab Global trial 2 countries	<ul style="list-style-type: none"> Primary endpoints: DLT, ORR Secondary endpoints: DoR, DCR, PFS, TTR, OS 	<ul style="list-style-type: none"> FPCD: Q2 2020
Phase Ib U105 NCT03523572	HER2-expressing breast and urothelial cancer	99	Non randomised sequential assignment • <i>Enhertu</i> + nivolumab Global trial 7 countries	<ul style="list-style-type: none"> Primary endpoints: DLT, ORR, TEAEs Secondary endpoints: DoR, DCR, PFS, TTR, OS, ORR (investigator) 	<ul style="list-style-type: none"> FPCD: Q3 2018 Data readout: Q3 2021



Calquence (BTK inhibitor)

Blood cancers

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase III ACE-CL-007 (ELEVATE-TN) NCT02475681	Previously untreated CLL	535	<ul style="list-style-type: none"> Arm A: chlorambucil + obinutuzumab Arm B: <i>Calquence</i> + obinutuzumab Arm C: <i>Calquence</i> 	<ul style="list-style-type: none"> 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) 	<ul style="list-style-type: none"> FPCD: Q2 2015 Data readout: Q2 2019 Primary endpoint met
Phase III ACE-CL-311 NCT03836261	Previously untreated CLL fit	780	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> + venetoclax Arm B: <i>Calquence</i> + venetoclax + obinutuzumab Arm C: FCR or BR 	<ul style="list-style-type: none"> Primary endpoint: IRC PFS (A vs C) Secondary endpoint: IRC PFS (B vs C); INV PFS (A vs C; B vs C) 	<ul style="list-style-type: none"> FPCD: Q1 2019 Data anticipated: 2023
Phase III ACE-CL-309 (ASCEND) NCT02970318	Relapsed/refractory CLL	306	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> Arm B: rituximab + idelalisib or bendamustine (investigator's choice) 	<ul style="list-style-type: none"> Primary endpoint: IRC assessed PFS (Arm A vs. Arm B) Secondary endpoints: INV-assessed ORR, OS, DoR, PROs 	<ul style="list-style-type: none"> FPCD Q3 2016 Data readout: Q2 2019 Primary endpoint met
Phase III ACE-CL-006 (ELEVATE-RR) NCT02477696	Relapsed/refractory high risk CLL	533	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> Arm B: ibrutinib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoints: comparison of incidence of infections, RTs (Richter's Transformation) and atrial fibrillation, OS 	<ul style="list-style-type: none"> FPCD: Q2 2015 Data readout: Q1 2021 Primary endpoint met
Phase III ACE-LY-308 (ECHO) NCT02972840	Previously untreated MCL	546	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> + bendamustine + rituximab Arm B: bendamustine + rituximab 	<ul style="list-style-type: none"> Primary endpoint: PFS by Lugano Classification for NHL Secondary endpoints: IA, PFS, ORR, DoR, time to response, OS 	<ul style="list-style-type: none"> FPCD: Q1 2017 Data anticipated: H2 2022
Phase III ESCALADE NCT04529772	DLBCL	600	<i>Calquence</i> + rituximab, cyclophosphamide, doxorubicin, vincristine, and prednisone	<ul style="list-style-type: none"> Primary endpoints: safety, ORR 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: 2023+
Phase II 15-H-0016 NCT02337829	Relapsed/refractory and treatment naïve/del17p CLL/SLL	48	<ul style="list-style-type: none"> <i>Calquence</i> monotherapy Arm A: lymph node biopsy Arm B: bone marrow biopsy 	<ul style="list-style-type: none"> Primary endpoint: ORR 	<ul style="list-style-type: none"> FPCD: Q4 2014 Data readout: Q4 2020

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Calquence (BTK inhibitor)

Blood cancers

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase I/II ACE-CL-001 NCT02029443	CLL/SLL/Richter's transformation	306	<i>Calquence</i> monotherapy Dose escalation and expansion	<ul style="list-style-type: none"> Primary endpoints: safety, PK, PD 	<ul style="list-style-type: none"> FPCD: Q1 2014 Data readout: Q4 2021
Phase Ib ACE-LY-106 NCT02717624	MCL	70	<i>Calquence</i> in combination with bendamustine and rituxumab <ul style="list-style-type: none"> Arm A: treatment naïve Arm B: relapsed/refractory Arm C: treatment naïve: <i>Calquence</i> + venetoclax + rituxumab 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q1 2016 Data anticipated: H2 2022
Phase I ACE-LY-003 NCT02180711	Relapsed/refractory follicular lymphoma	80	<ul style="list-style-type: none"> Arm A: <i>Calquence</i> Arm B: <i>Calquence</i> + rituximab Arm C: <i>Calquence</i> + rituximab + lenolidomide 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q1 2015 Data anticipated: 2023+
Phase I ACE-CL-003 NCT02296918	CLL/SLL/PLL	69	<i>Calquence</i> + obinutuzumab <ul style="list-style-type: none"> Arm A: relapsed/refractory Arm B: treatment naïve <i>Calquence</i> + venetoclax + rituxumab <ul style="list-style-type: none"> Arm C: relapsed/refractory Arm D: treatment naïve 	<ul style="list-style-type: none"> Primary endpoints: safety, ORR Secondary endpoints: PD, PFS, TTNT, OS 	<ul style="list-style-type: none"> FPCD: Q4 2014 Data anticipated: H1 2022

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Calquence (BTK inhibitor)

Blood and other cancers

Trial	Population	Patients	Design	Endpoint(s)	Status
Phase I/II D8220C0007 NCT03932331	Chinese adults R/R MCL and R/R CLL	105	Part 1: R/R B-cell Malignancies Part 2: Cohort A: R/R MCL Part 2: Cohort B: R/R CLL	<ul style="list-style-type: none"> Primary endpoints: safety, ORR 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: MCL H1 2022 Data anticipated: CLL 2023+
Phase I NCT03198650	Japanese adults with advanced B-cell malignancies	34	Dose confirmation and expansion <ul style="list-style-type: none"> Calquence Dose confirmation <ul style="list-style-type: none"> Calquence + obinutuzumab 	<ul style="list-style-type: none"> Primary endpoints: safety, PK 	<ul style="list-style-type: none"> FPCD: Q2 2017 Data anticipated: H2 2022
Phase I D8220C00018 NCT04488016	Healthy volunteers	28	<ul style="list-style-type: none"> Part 1: Rel bioavailability for capsule vs. tablet Part 2: Rel bioavailability for oral solution of tablet 	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data readout: Q1 2021
Phase I D8223C00013 NCT04768985	Healthy volunteers	66	<ul style="list-style-type: none"> Arm A: Calquence tablet Arm B: Calquence capsule 	<ul style="list-style-type: none"> Primary endpoint: bioequivalence 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data readout: Q4 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: <i>Koselugo</i> 25mg/m ² BID with 2 strata: <ul style="list-style-type: none"> Stratum 1: PN related morbidity present at enrolment Stratum 2: no PN related morbidity present at enrolment 	<ul style="list-style-type: none"> Primary endpoint: Complete partial and complete response rate measured by volumetric MRI, Duration of response and functional outcomes/QoL 	<ul style="list-style-type: none"> FPCD: Q3 2015 LPCD: Q4 2016 Data readout: Q1 2019 Primary endpoint met
Phase I Japan PK / Safety trial NCT04495127 Partnered	Paediatric inoperable NF1-PN patients	9-12	Open-label trial <ul style="list-style-type: none"> <i>Koselugo</i> in Japanese paediatric NF1-PN patients 	<ul style="list-style-type: none"> Primary endpoint: safety Secondary endpoints: PK, anti-tumour effect 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2020 Data readout: Q4 2021
Phase I China PK / Safety / Efficacy trial NCT04590235	Pediatric (2-17 years old), adult NF1	32	Single arm trial with 3 phases; Dose confirmation phase (n=6 for 3 cycles), Expansion phase (24mths post LSD) Long term follow up (60mths post LSD)	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability and PK Secondary endpoints: efficacy (ORR, DoR; TTR; PFS) 	<ul style="list-style-type: none"> FPCD: Q4 2020
Phase I <i>Koselugo</i> with a low-fat meal compared to fasted state NCT05101148	Adolescents aged ≥ 12 to < 18 years at trial entry with a clinical diagnosis of NF1 related PN.	20 to be enrolled (to achieve 16 evaluable participants completing T2)	Single-arm, multiple dose, sequential, two or three period trial <ul style="list-style-type: none"> <i>Koselugo</i> 25mg/m² BID given with a low-fat meal versus the same dose given in a fasted state. 	<ul style="list-style-type: none"> Primary endpoints: PK (steady state systemic exposure), safety (especially GI toxicity) 	<ul style="list-style-type: none"> FPCD: Q3 2021
Phase III KOMET NCT04924608	Adult age ≥18 years with NF1 who have symptomatic, inoperable PN. Available baseline chronic target PN pain score	146	Multicentre, international study with a parallel, randomized, double blind, placebo controlled, 2 arm design <ul style="list-style-type: none"> Arm A: <i>Koselugo</i> 25mg/m² BID Arm B: Placebo BID until end of Cycle 12, then cross over to <i>Koselugo</i> 25mg/m² BID 	<ul style="list-style-type: none"> Primary Endpoint: ORR by end of Cycle 16 on <i>Koselugo</i> vs placebo as determined by ICR per REiNS criteria. Key secondary endpoint: Chronic PN-pain intensity (change from baseline) on <i>Koselugo</i> vs placebo 	<ul style="list-style-type: none"> FPCD Q4 2021 Data anticipated: 2023+



Orpathys (savolitinib, MET inhibitor)

NSCLC and other cancers

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT02897479 Partnered	Lung PSC and other NSCLC	65	Single arm trial • Orpathys QD China	<ul style="list-style-type: none">Primary endpoint: ORRSecondary endpoints: PFS, safety parameters	<ul style="list-style-type: none">FPCD: Q1 2017Data readout: Q2 2020
Phase I NCT01985555 Partnered	Advanced NSCLC (all comers)	85	Dose escalation trial China	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK profile	<ul style="list-style-type: none">FPCD: Q2 2013Data readout: Q3 2019

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Capivasertib (AKT inhibitor)

Breast cancer, prostate cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAPItello-290 NCT03997123	Locally advanced or metastatic TNBC	924	Double-blind randomised comparative trial <ul style="list-style-type: none"> Arm 1: capivasertib + paclitaxel Arm 2: placebo + paclitaxel 	<ul style="list-style-type: none"> Primary endpoint: OS 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: 2023
Phase III CAPItello-291 NCT04305496	2L and beyond in AI resistant locally advanced (Inoperable) or metastatic HR+/HER2- breast cancer	834	Double-blind randomised comparative trial <ul style="list-style-type: none"> Arm 1: capivasertib + <i>Faslodex</i> Arm 2: placebo + <i>Faslodex</i> 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD: Q2 2020 Data anticipated: 2023
Phase III CAPItello-281 NCT04493853	De novo PTEN deficient metastatic hormone sensitive prostate cancer	1000	Double-blind randomised comparative trial <ul style="list-style-type: none"> Arm 1: capivasertib + abiraterone Arm 2: placebo + abiraterone 	<ul style="list-style-type: none"> Primary endpoint: rPFS 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: 2023+
Phase III CAPItello-292 NCT04862663	1L triplet in early relapse/endocrine-resistant locally advanced (inoperable) or metastatic HR+/HER2- breast cancer	700	Double-blind randomised comparative trial <ul style="list-style-type: none"> Arm 1: capivasertib + palbociclib + <i>Faslodex</i> Arm 2: placebo + palbociclib + <i>Faslodex</i> 	<ul style="list-style-type: none"> Primary endpoint: PFS 	<ul style="list-style-type: none"> FPCD Q2 2021 Data anticipated 2023+



Monalizumab (NKG2a mAb)

Cancers

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III INTERLINK-1 NCT04590963	Recurrent or metastatic HNSCC, 2L	600	<ul style="list-style-type: none"> Arm A: monalizumab + cetuximab i.v. Arm B: placebo + cetuximab i.v. <p>Global</p>	<ul style="list-style-type: none"> Primary: OS Secondary: PFS, ORR, DoR 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023+
Phase I/II NCT02671435	Advanced solid tumours	381	<p>Escalation phase</p> <ul style="list-style-type: none"> monalizumab + <i>Imfinzi</i> i.v. <p>Expansion phase</p> <ul style="list-style-type: none"> monalizumab + <i>Imfinzi</i> i.v. recommended dose <p>Exploration phase</p> <ul style="list-style-type: none"> monalizumab + <i>Imfinzi</i> 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 <p>Global</p>	<ul style="list-style-type: none"> Primary endpoints: safety Exploration phase primary endpoint: Objective Response per RECIST Secondary endpoints include: tumour response (OR, DC, DoR, PFS and OS), immunogenicity, pharmacokinetics, pharmacodynamics 	<ul style="list-style-type: none"> FPCD: Q2 2016 Data readout: Q4 2021

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Camizestrant (AZD9833, next generation oral SERD)

Breast cancer

Trial	Population	Patients	Design	Endpoints	Status
Phase III SERENA-4 NCT04711252	HR+ HER2- breast cancer	1342	Randomised, double-blind, comparative trial <ul style="list-style-type: none"> Arm A: camizestrant (AZD9833) + palbociclib Arm B: anastrozole + palbociclib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS, PFS2 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase III SERENA-6 NCT04964934	HR+ HER2- breast cancer	300	Randomised, double-blind, comparator trial <ul style="list-style-type: none"> Arm A: camizestrant (AZD9833) plus palbociclib or abemaciclib Arm B: anastrozole or letrozole plus palbociclib or abemaciclib 	<ul style="list-style-type: none"> Primary endpoint: PFS Secondary endpoint: OS, PFS2 	<ul style="list-style-type: none"> FPCD Q3 2021 Data anticipated: 2023
Phase II SERENA-2 NCT04214288	HR+ breast cancer	240	Randomised, open-label, parallel-group, multicentre trial <ul style="list-style-type: none"> camizestrant (AZD9833) vs. i.m. <i>Faslodex</i> in women with advanced breast cancer. 	<ul style="list-style-type: none"> Primary outcome: mPFS 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q3 2021 Data anticipated: H2 2022
Phase II SERENA-3 NCT04588298	HR+ breast cancer	84	Randomised, open-label, parallel-group, multicentre trial	<ul style="list-style-type: none"> Primary outcome: change in ER expression between pre- and on-treatment tumour biopsies 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023
Phase I NCT04541433	HR+ breast cancer	18	Open-label <ul style="list-style-type: none"> anti-tumour activity of camizestrant (AZD9833) in Japanese women with endocrine resistant HR+ HER2- breast cancer that is not amenable to treatment with curative intent. 	<ul style="list-style-type: none"> Primary outcomes: safety and tolerability Secondary outcome: PK 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023
Phase I SERENA-1 NCT03616587	HR+ breast cancer	340	Escalation phase - open label multicentre trial <ul style="list-style-type: none"> camizestrant (AZD9833) camizestrant (AZD9833) + palbociclib, everolimus, abemeciclib or capivasertib. Expansion phase - randomised expansion cohort(s) <ul style="list-style-type: none"> camizestrant (AZD9833) camizestrant (AZD9833) + palbociclib, everolimus, abemeciclib or capivasertib. 	<ul style="list-style-type: none"> Primary outcomes: safety and tolerability Secondary outcomes: PK, antitumour activity 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: 2023
Phase I NCT04546347	Healthy volunteers	32	Randomised, open-label	<ul style="list-style-type: none"> Primary outcome: relative bioavailability of different tablet formulations and the effect of food 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2020 Data readout: Q3 2021
Phase I NCT04818632	HR+ HER2- breast cancer Chinese patients	42	Dose escalation <ul style="list-style-type: none"> camizestrant (AZD9833) Dose expansion <ul style="list-style-type: none"> camizestrant (AZD9833) camizestrant (AZD9833) + palbociclib camizestrant (AZD9833) + everolimus 	<ul style="list-style-type: none"> Primary outcomes: safety and tolerability, PK Secondary outcome: antitumour activity 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023



Datopotamab deruxtecan (TROP2 ADC)

NSCLC

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Lung01 NCT04656652 Partnered	Previously treated advanced or metastatic NSCLC without actionable genomic alterations	590	Randomised, open label, parallel assignment <ul style="list-style-type: none"> Arm 1: datopotamab deruxtecan Arm 2: docetaxel Global	<ul style="list-style-type: none"> Primary endpoints: PFS, OS Secondary endpoints: ORR, DoR, TTR, DCR, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023
Phase III TROPION-Lung08 Partnered	Treatment-naïve patients with PD-L1 high advanced or metastatic NSCLC without actionable genomic alterations	740	Randomised, open label <ul style="list-style-type: none"> Arm 1: datopotamab deruxtecan + pembrolizumab Arm 2: pembrolizumab Global	<ul style="list-style-type: none"> Primary endpoints: PFS, OS 	<ul style="list-style-type: none"> Initiating Data anticipated: 2023+
Phase II TROPION-Lung05 NCT04484142 Partnered	Advanced or metastatic NSCLC with actionable genomic alterations and progressed on or after kinase inhibitor therapy and platinum-based chemotherapy	150	Single-arm, open label <ul style="list-style-type: none"> datopotamab deruxtecan Global trial	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DOR, PFS, OS, safety, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase I TROPION-Lung02 NCT04526691 Partnered	Advanced or metastatic NSCLC	120	Open label, two-part (dose escalation, dose expansion), sequential assignment <ul style="list-style-type: none"> datopotamab deruxtecan + pembrolizumab +/- platinum chemotherapy US, Japan	<ul style="list-style-type: none"> Primary endpoints: DLT, safety Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023+
Phase I TROPION-Lung04 NCT04612751 Partnered	Advanced or metastatic NSCLC	120	Open label, two-part (dose escalation, dose expansion), sequential assignment <ul style="list-style-type: none"> datopotamab deruxtecan + <i>Imfinzi</i> +/- platinum chemotherapy US, Japan	<ul style="list-style-type: none"> Primary endpoints: DLT, safety Secondary endpoints: ORR, DOR, PFS, OS, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+



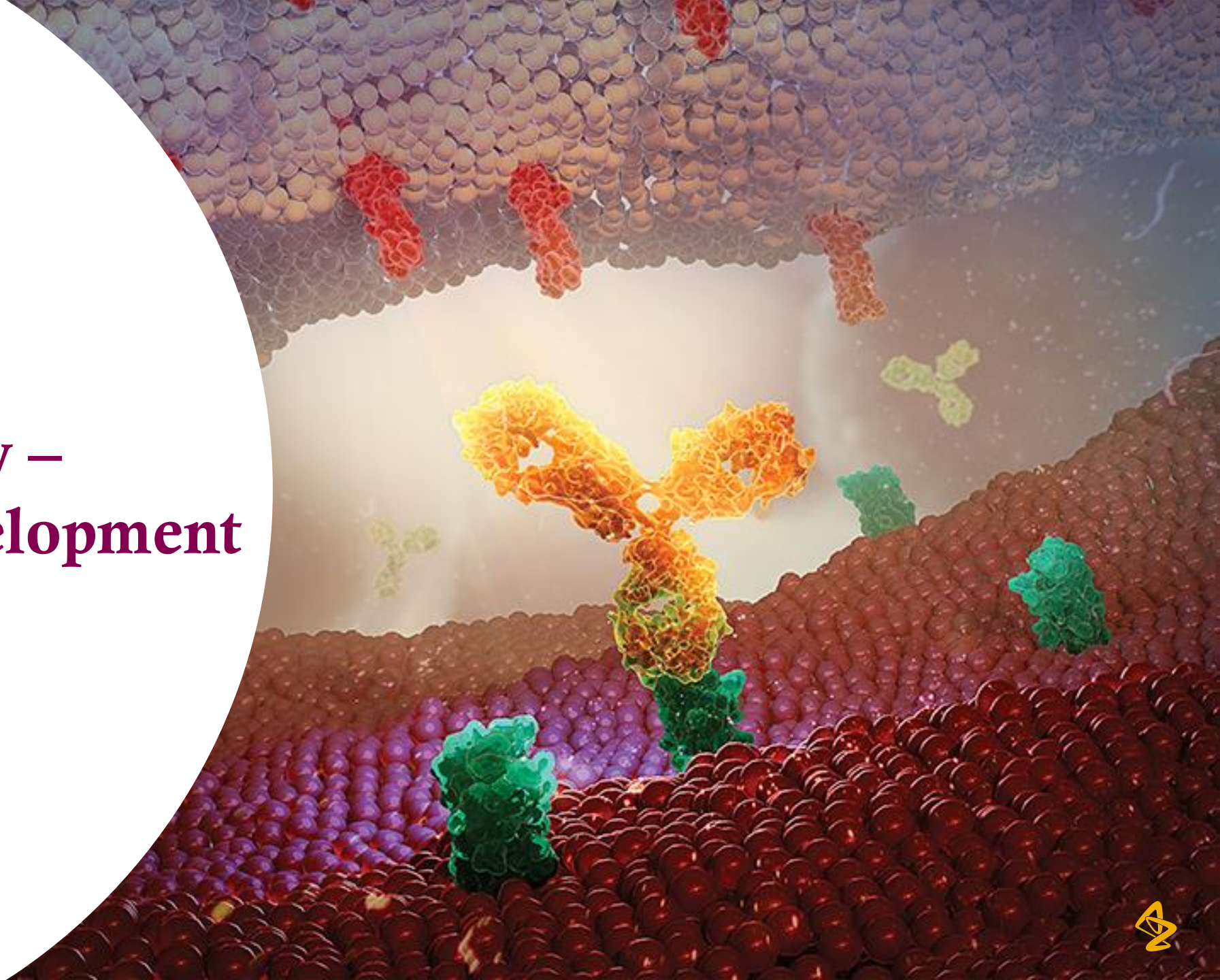
Datopotamab deruxtecan (TROP2 ADC)

NSCLC and other cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase III TROPION-Breast01 NCT05104866	Inoperable or metastatic HR+, HER2- breast cancer	700	Open-label, randomized <ul style="list-style-type: none"> • datopotamab deruxtecan • investigator's choice standard of care chemotherapy (eribulin, vinorelbine, capecitabine, gemcitabine) 	<ul style="list-style-type: none"> • Primary endpoints: PFS, OS • Secondary endpoints: ORR, DoR, DCR, PL, ADA 	<ul style="list-style-type: none"> • FPCD: Q4 2021 • Data anticipated: 2023+
Phase I TROPION-PanTumor01 NCT03401385 Partnered	Subjects with advanced solid tumours NSCLC TNBC HR+ BC HER2-negative gastric/GEJ oesophageal Urothelial SCLC	770	Open label, two-part (dose escalation, dose expansion), sequential assignment <ul style="list-style-type: none"> • datopotamab deruxtecan Japan, US	<ul style="list-style-type: none"> • Primary endpoints: DLT, safety • Secondary endpoints: PK, anti-tumour activity, ADA 	<ul style="list-style-type: none"> • FPCD: Q1 2018 • Data anticipated: 2023+ • Early data readout (NSCLC): Q1 2021 • Early data readout (TNBC): Q2 2021



**Oncology –
early-stage development**



AZD0466 (Bcl2/xL inhibitor)

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT04865419	Advanced haematologic malignancies	160	Module 1 Part A: Dose escalation • AZD0466 Part B: Dose expansion • AZD0466 Module 2 - DDI trial AZD0466 with voriconazole	<ul style="list-style-type: none">Primary endpoint: safetySecondary endpoint: PK	<ul style="list-style-type: none">FPCD: Q2 2021Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



MEDI1191 (IL-12 modRNA)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03946800	Advanced solid tumours	87	Open-label, dose-escalation and expansion trial of MEDI1191 administered intratumourally as monotherapy and in combination with <i>Imfinzi</i>	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK, immunogenicity and efficacy	<ul style="list-style-type: none">FPCD: Q2 2019Data anticipated: 2023



AZD1390 (ATM inhibitor)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

Trial	Population	Subjects	Design	Endpoints	Status
Phase I NCT03423628	Recurrent glioblastoma eligible for re-irradiation, brain metastases and leptomeningeal disease, newly-diagnosed glioblastoma patients	Up to 162	Open label trial <ul style="list-style-type: none">Arm A: recurrent GBM, AZD1390 +RT in dose escalation cohortsArm C: primary GBM, AZD1390 +RT in dose escalation cohorts	<ul style="list-style-type: none">Primary endpoints: safety, tolerability, MTDSecondary endpoints: PK and preliminary assessment of anti-tumour activity	<ul style="list-style-type: none">FPCD: Q2 2018Data anticipated: 2023

CVRM

R&I

Other

COVID-19

Rare Disease



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	<ul style="list-style-type: none"> Arm B: paclitaxel + adavosertib Arm C: carboplatin + adavosertib Global trial	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, PFS, OS, DCR, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2015 LPCD: Q2 2018 Data readout: Q3 2019
Phase II D601HC00002 NCT04590248	Uterine serous carcinoma	120	<ul style="list-style-type: none"> adavosertib monotherapy Phase IIb, open-label, single-arm, multicentre study Global trial	<ul style="list-style-type: none"> Primary endpoint: ORR Secondary endpoints: DoR, depth of response, PFS 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022
Phase I D6015C00002 NCT02617277	Advanced solid tumours	56	Dose escalation trial <ul style="list-style-type: none"> adavosertib + <i>Imfinzi</i> US	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, MTD 	<ul style="list-style-type: none"> FPCD: Q4 2015 LPCD: Q4 2018 Data readout: Q4 2019



AZD4573 (CDK9 inhibitor)

Blood cancers

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT04630756	R/R haematologic malignancies	78	Open label, non-randomised trial Module 1 Part A: Dose setting <ul style="list-style-type: none"> AZD4573 + <i>Calquence</i> (100mg twice daily) combination in DLBCL, all comers; ramp-up across 3 dose levels Module 1 Part B: Dose expansion <ul style="list-style-type: none"> AZD4573 + <i>Calquence</i> (100mg twice daily) combination in GCB and non-GCB DLBCL Module 2 Part A: Dose Confirmation <ul style="list-style-type: none"> AZD4573 monotherapy window followed by AZD4573 + acalabrutinib in patients with relapsed or refractory MCL i.v. route of administration 10 countries across North America, EU, ROW	<ul style="list-style-type: none"> Primary endpoint Part A: safety Primary endpoint Part B: ORR Secondary endpoints Part A: safety, anti-tumour activity, PK 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase I NCT03263637	R/R haematologic malignancies	45	<ul style="list-style-type: none"> Arm 1: dose escalation in haematological malignancies excluding AML/ALL/high-risk MDS/CMML/CLL Arm 2: dose escalation in relapsed or refractory AML, ALL, high-risk MDS, CMML, CLL and Richter's syndrome i.v. route of administration Open in Netherlands, UK, Germany	<ul style="list-style-type: none"> Primary endpoints: safety, PK Secondary endpoint: efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q3 2021 Data anticipated: H2 2022
Phase II NCT05140382	R/R Peripheral T-cell lymphoma and R/R classical Hodgkins Lymphoma	90	Open label, non-randomised modular dose confirmation and expansion study in pts with relapsed/ refractory PTCL or cHL Module 1: Exploring AZD4573 monotherapy treatment in 3 cohorts: <ul style="list-style-type: none"> Cohort 1: PTCL, all comers (excluding NKTCL) Cohort 2: PTCL (NKTCL only) Cohort 3: cHL i.v. Route of administration ~30 centres in 10 countries across North America, Europe, EU and ROW	<ul style="list-style-type: none"> Primary endpoints: Efficacy Secondary endpoint: Safety, PK 	<ul style="list-style-type: none"> FPCD Q1 2022 Data anticipated: 2023



AZD5305 (PARP1 inhibitor)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa NCT04644068	Advanced, metastatic HER2- breast cancer (BRCAm, PALB2m or RAD51C/Dm) Advanced, metastatic TNBC PSR ovarian cancer (BRCAm, PALB2m or RAD51C/Dm) PSR ovarian cancer (HRD+) Prostate (mCRPC, BRCAm) Prostate (mCRPC, HRRm)	715	A modular, open-label, multicentre trial dose escalation <ul style="list-style-type: none"> Module 1: AZD5305 Module 2: AZD5305 + paclitaxel Module 3: AZD5305 + carboplatin +/- paclitaxel Module 4: AZD5305 + trastuzumab deruxtecan Module 5: AZD5305 + datopotamab deruxtecan 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK Secondary endpoints: efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023+



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

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa NCT03530397	Advanced solid tumours	366	Open-label, dose-escalation and dose-expansion trial Dose escalation: MEDI5752 i.v. Dose expansion: MEDI5752 i.v. as monotherapy and in combination with chemotherapy <ul style="list-style-type: none"> Arm A: MEDI5752 i.v. Arm B: MEDI5752 i.v., pemetrexed and carboplatin Arm C: pembrolizumab, pemetrexed and carboplatin Arm D: MEDI5752 i.v., taxane (paclitaxel or nab-paclitaxel) and carboplatin 	<ul style="list-style-type: none"> Dose escalation primary endpoints: safety, MTD Dose expansion primary endpoint: antitumour activity based on OR Secondary endpoints: PK, ADA, tumoral baseline PD-L1, antitumour activity (OR, DoR, DCR, PFS, OS) 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data anticipated: 2023+
Phase Ib NCT04522323	Advanced renal cell carcinoma	70	Open-label, dose-escalation and dose-expansion trial <ul style="list-style-type: none"> Arm 1: MEDI5752 and axitinib Arm 2: MEDI5752 and lenvatanib 	<ul style="list-style-type: none"> Dose escalation primary endpoints: safety, MTD, RP2D & tolerability. Assess antitumour activity of the combination (ORR) Secondary endpoints: PK, ADA and antitumour activity (PFS, OR, DoR, DCR, TTR, OS) 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



AZD5991 (MCL1 inhibitor)

Blood cancers

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I/Ib/IIa NCT03218683	Relapsed/refractory haematologic malignancies	144	<ul style="list-style-type: none">• Arm 1: AZD5991 dose escalation and expansion in R/R haematological malignancies• Arm 2: AZD5991 + venetoclax combination dose escalation in R/R AML i.v. route of administration US only	<ul style="list-style-type: none">• Primary endpoint: safety• Secondary endpoints: PK, efficacy	<ul style="list-style-type: none">• FPCD: Q3 2017• Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Ceralasertib (AZD6738, ATR inhibitor)

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II PLANETTE NCT04564027	Solid tumours mCRPC	52	<ul style="list-style-type: none"> Cohort A: ceralasertib; ATM-altered AST Cohort B: ceralasertib; ATM-altered mCRPC 	<ul style="list-style-type: none"> Cohort A primary endpoint: ORR Cohort B primary endpoint: Composite RR 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase I NCT02264678	Solid tumours	250	<ul style="list-style-type: none"> Arm 1: ceralasertib + carboplatin Arm 2: ceralasertib dose escalation, ceralasertib + <i>Lynparza</i> Arm 3: ceralasertib + <i>Imfinzi</i> North America, Europe and South Korea	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK and efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2014 Data anticipated: 2023+
Phase I NCT03022409	HNSCC	44	Window of opportunity <ul style="list-style-type: none"> Arm 1: ceralasertib Arm 2: <i>Lynparza</i> US, France, Taiwan and the UK	<ul style="list-style-type: none"> Primary endpoint: Biomarker change 	<ul style="list-style-type: none"> FPCD: Q4 2017 Data anticipated: Q4 2021

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



AZD7648 (selective DNA-PK inhibitor)

Advanced solid tumours

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03907969	Advanced malignancies	234	Modular dose escalation and dose expansion trial <ul style="list-style-type: none">• Arm 1: AZD7648 monotherapy• Arm 2: AZD7648 + pegylated liposomal doxorubicin US, UK	<ul style="list-style-type: none">• Primary outcomes: safety and tolerability• Secondary outcomes: PK, Cytochrome P450, preliminary anti-tumour activity	<ul style="list-style-type: none">• FPCD: Q4 2019• Data anticipated: 2023+



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 <ul style="list-style-type: none">Arm 1: AZD8701 monotherapyArm 2: AZD8701 & <i>Imfinzi</i> combination therapy i.v. route of administration US, CA, FR, ES	<ul style="list-style-type: none">Primary endpoints: safety & tolerabilitySecondary endpoints: PK, PD, preliminary anti-tumour activity	<ul style="list-style-type: none">FPCD: Q3 2020Data anticipated: 2023+



MEDI9253 (rNDV-IL-12)

Solid tumours

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04613492	Advanced solid tumours	86	Open-label, dose-escalation and expansion trial <ul style="list-style-type: none">MEDI9253 + <i>Imfinzi</i>	<ul style="list-style-type: none">Primary endpoint: safety and tolerabilitySecondary endpoints: PK, PD, immunogenicity and efficacy	<ul style="list-style-type: none">FPCD: Q4 2020Data anticipated: 2023+



IPH5201 (CD39 mAb)

Solid tumours

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04261075 Partnered	Advanced solid tumours	204	Open-label, dose-escalation trial to determine MTD of IPH5201 as monotherapy, or in combination with <i>Imfinzi</i> +/- oleclumab. <ul style="list-style-type: none">Part 1: IPH5201 monotherapy dose escalation to MTDPart 2: IPH5201 + <i>Imfinzi</i> dose escalation to MTDPart 3: IPH5201 + <i>Imfinzi</i> + oleclumab dose escalation to MTD Route of administration: i.v. 4 countries - US and 3 in EU.	<ul style="list-style-type: none">Primary endpoints: AE, SAE, DLTSecondary endpoints: OR, DC, PK, ADA	<ul style="list-style-type: none">FPCD: Q1 2020Data anticipated: 2023



Oleclumab (CD73 mAb)

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase Ib/II NCT03611556	Pancreatic 1L and 2L with prior gemcitabine-based chemotherapy	339	<ul style="list-style-type: none"> • 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. + <i>Imfinzi</i> i.v. <p>US, Norway, Spain and Australian trial centres</p>	<ul style="list-style-type: none"> • Primary endpoints: safety and anti-tumour activity • Secondary endpoints include: PFS, PK, immunogenicity, safety and anti-tumour activity 	<ul style="list-style-type: none"> • FPCD: Q2 2018 • Data anticipated: 2023

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



AZD2936 (PD-1/TIGIT Bispecific mAb)

Solid tumours

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04995523 Partnered	NSCLC	147	<p>Open-label, non-randomised dose-escalation and dose-expansion trial:</p> <ul style="list-style-type: none"> Part A: Dose escalation in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 Intravenous monotherapy Part B: Dose expansion in checkpoint inhibitor (CPI) experienced NSCLC pts with AZD2936 intravenous monotherapy Part C: Dose expansion in CPI Naive NSCLC pts with AZD2936 i.v. monotherapy Part D: Dose expansion. Design to be confirmed by protocol amendment <p>Europe, Australia, South Korea and North America</p>	<ul style="list-style-type: none"> Part A Dose escalation primary endpoints: safety, RP2D, MTD Part B dose expansion primary endpoints: Safety and efficacy (ORR) Part C dose expansion primary endpoints: Safety and efficacy (ORR) Secondary endpoints: PK, PD (Receptor occupancy), efficacy (DCR, DoR, DRR, PFS) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+



AZD7789 (PD-1/TIM3 Bispecific mAb)

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I/IIa NCT04931654	NSCLC Other tumours	81	Open-label, non-randomised dose-escalation and dose-expansion trial: <ul style="list-style-type: none">Part A: Dose escalation in post IO NSCLC pts with AZD7789 intravenous (iv) monotherapyPart B: Dose expansion in post IO and IO naïve NSCLC pts with AZD7789 iv monotherapy. North America, Europe	<ul style="list-style-type: none">Primary endpoints: AE, SAE, DLTs, ORRSecondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA	<ul style="list-style-type: none">FPCD: Q4 2021Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



AZD8205 (B7H4 ADC)

Cancer

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II NCT05123482	Breast Cancer, Cholangiocarcinoma, Ovarian Cancer, Endometrial Cancer	198	Open-label, non-randomised dose-escalation and dose-expansion trial	<ul style="list-style-type: none">Primary endpoints: AE, SAE, DLTs, Changes in Lab and PE parametersSecondary endpoints: ORR, DCR, DoR, PFS, OS, PK, ADA	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: 2023+



AZD0171 (anti-LIF mAb)

Cancer

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04999969	1L metastatic pancreatic ductal adenocarcinoma	115	Open-label, non-randomised trial of AZD0171 + Imfinzi + gemcitabine, nab-paclitaxel	<ul style="list-style-type: none">Primary endpoints: Safety, OS at 12mSecondary endpoints: ORR, DoR, PFS	<ul style="list-style-type: none">FPCD: Q1 2022Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



**BioPharmaceuticals –
approved medicines and
late-stage pipeline**



Farxiga (SGLT2 inhibitor)

Heart failure and chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III DELIVER NCT03619213	CHF patients with HFpEF	6100	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 10mg QD Arm 2: placebo Global trial - 21 countries	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: H1 2022
Phase III DAPA-MI NCT04564742	Patients with myocardial infarction	6400	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 10mg QD Arm 2: placebo Global trial - 2 countries	<ul style="list-style-type: none"> Primary endpoint: time to the first occurrence of any of the components of the composite: hospitalisation for HF or CV death 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023
Phase I NCT04856007	Healthy Chinese volunteers	80	<ul style="list-style-type: none"> Arm 1: <i>Farxiga</i> 5 mg + metformin 500 mg XR Arm 2: <i>Farxiga</i>/metformin XR FDC 5/500 mg Arm 3: <i>Farxiga</i> 10 mg + metformin 1000 mg XR Arm 4: <i>Farxiga</i>/metformin XR FDC 10/1000 mg China only	<ul style="list-style-type: none"> Primary endpoint: Plasma AUCinf, AUClast and Cmax of <i>Farxiga</i> and metformin respectively. 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q2 2021 Data readout: Q4 2021



Lokelma (sodium zirconium cyclosilicate)

Hyperkalaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase IIIb DIALIZE China NCT04217590	Patients with ESRD with hyperkalaemia and on stable haemodialysis	134	<ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> 5g QD for 8 weeks on non-dialysis days. Option to uptitrate to 10 and 15g QD. Arm 2: placebo QD for 8 weeks on non-dialysis days China	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H1 2022
Phase III HARMONIZE Asia NCT03528681	Hyperkalaemia	250	Open-label <i>Lokelma</i> 10g TID for 48 hours followed by: <ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> 5g QD for 28 days Arm 2: <i>Lokelma</i> 10g QD for 28 days Arm 3: placebo QD for 28 days China	<ul style="list-style-type: none"> Primary endpoint: maintenance of normokalaemia 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H2 2022
Phase III DIALIZE-Outcomes NCT04847232	Patients with recurrent hyperkalaemia on chronic haemodialysis	2300	<ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> 5g-15g QD for 4 weeks on non-dialysis days, thereafter adjusted monthly Arm 2: placebo QD Global trial – 22 countries	<ul style="list-style-type: none"> Primary endpoint: Time to first occurrence of SCD, stroke, or hospitalisation/intervention/ED visit due to arrhythmias 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+
Phase III STABILIZE-CKD NCT05056727	Patients with CKD and hyperkalaemia or at risk of hyperkalaemia	1360	Open-label <i>Lokelma</i> (10g TID or 5g QD) for up to 72h, followed by 3 months open-label treatment with <i>Lokelma</i> (5g QOD to 15g QD) and uptitration of lisinopril or valsartan. Thereafter, patients are randomized to a 24 month treatment: <ul style="list-style-type: none"> Arm 1: <i>Lokelma</i> (5g QOD to 15g QD) and lisinopril or valsartan Arm 2: Placebo and lisinopril or valsartan Global trial – 13 countries	<ul style="list-style-type: none"> Primary endpoint: Total slope (eGFR measurements starting at randomisation) and chronic slope (eGFR measurements starting at 12 weeks after randomisation) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+



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	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: placebo Global trial	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q4 2012 LPCD: Q3 2018 Data readout: Q4 2018 Primary endpoint met Sponsored by FibroGen
Phase III ALPS NCT01887600 Partnered	Anaemia in CKD in patients not receiving dialysis	597	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: placebo Global trial	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q2 2013 LPCD: Q4 2017 Data readout: Q3 2018 Primary endpoint met Sponsored by Astellas
Phase III DOLOMITES NCT02021318 Partnered	Anaemia in CKD in patients not receiving dialysis	616	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: darbepoetin alfa Global trial	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q1 2014 LPCD: Q4 2019 Data readout: Q1 2020 Primary endpoint met Sponsored by Astellas
Phase III OLYMPUS NCT02174627	Anaemia in CKD in patients not receiving dialysis	2781	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: placebo Global trial	<ul style="list-style-type: none"> Primary efficacy endpoint: haemoglobin response Primary safety objective: contribute CV safety data to pooled safety analyses across the Phase III program 	<ul style="list-style-type: none"> 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	2133	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: epoetin alfa Global trial	<ul style="list-style-type: none"> Primary efficacy endpoint: haemoglobin response Primary safety objective: contribute CV safety data to pooled safety analyses across the Phase III programme 	<ul style="list-style-type: none"> FPCD: Q3 2014 LPCD: Q3 2018 Data readout: Q4 2018 Primary endpoint met Sponsored by AstraZeneca



Roxadustat (HIF-PH inhibitor)

Anaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase III SIERRAS NCT02273726 Partnered	Anaemia in CKD in patients receiving dialysis	741	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: epoetin alfa Global trial	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q4 2014 LPCD: Q3 2018 Data readout: Q4 2018 Primary endpoint met Sponsored by FibroGen
Phase III PYRENEES NCT02278341 Partnered	Anaemia in CKD in patients receiving dialysis	838	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: epoetin alfa or darbepoetin alfa Global trial	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q4 2014 LPCD: Q3 2018 Data readout: Q3 2018 Primary endpoint met Sponsored by Astellas
Phase III HIMALAYAS NCT02052310 Partnered	Anaemia in newly initiated dialysis patients	1043	<ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: epoetin alfa Global trial	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> 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 <ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: placebo US/global trial	<ul style="list-style-type: none"> Primary endpoint: proportion of patients achieving transfusion independence 	<ul style="list-style-type: none"> FPCD: Q3 2017 Data anticipated: 2023 Sponsored by FibroGen
Phase II/III NCT03303066 Partnered	Anaemia in lower risk MDS patients	175	Open label roxadustat lead-in <ul style="list-style-type: none"> Arm 1: roxadustat Arm 2: placebo China	<ul style="list-style-type: none"> Primary endpoint: haemoglobin response 	<ul style="list-style-type: none"> FPCD: Q2 2018 Data anticipated: 2023 Sponsored by FibroGen
Phase II NCT04076943 Partnered	Anaemia in patients receiving chemotherapy treatment for non-myeloid malignancies	92	Open label trial <ul style="list-style-type: none"> roxadustat 3x week 16 weeks US	<ul style="list-style-type: none"> Primary endpoint: maximum change in haemoglobin within 16 weeks from baseline without RBC transfusion 	<ul style="list-style-type: none"> FPCD: Q3 2019 LPCD: Q3 2020 Data readout: Q2 2021 Primary endpoint met Sponsored by FibroGen



AZD4831 (MPO inhibitor)

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb/ Phase III NCT04986202	HFpEF	1485	Randomised, double-blind <ul style="list-style-type: none"> Arm 1: 2.5mg AZD4831 Arm 2: 5mg AZD4831 Arm 3: placebo Global trial	<ul style="list-style-type: none"> Endpoints: Efficacy and Safety 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+
Phase IIa NCT03756285	HFpEF	96	<ul style="list-style-type: none"> Arm 1: AZD4831 Arm 2: placebo Global trial – five countries	<ul style="list-style-type: none"> Primary endpoint: The change from baseline in MPO activity in % 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data readout: Q2 2021
Phase I NCT02712372	Healthy patients	c. 96	SAD trial <ul style="list-style-type: none"> Arm 1: AZD4831 Arm 2: placebo Germany	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q3 2016 LPCD: Q4 2016 Data readout: Q2 2017
Phase I NCT03136991	Healthy patients	c. 40	MAD trial <ul style="list-style-type: none"> Arm 1: AZD4831 Arm 2: placebo USA	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoint: PK parameters 	<ul style="list-style-type: none"> FPCD: Q2 2017 LPCD: Q4 2017 Data readout: Q1 2018
Phase I NCT04232345	Healthy patients	32	MAD trial in Japanese and Chinese patients	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD Q1 2020 Data readout: Q3 2021
Phase I NCT04407091	Healthy patients	6	Open label hADME trial <ul style="list-style-type: none"> a single oral dose of [14C] AZD4831 UK	<ul style="list-style-type: none"> Primary endpoints: mass balance, with routes and rates of elimination of [14C]AZD4831; Metabolite profiling and structural identification; PK and total radioactivity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q3 2020 Data readout: Q3 2021
Phase I NCT05052710	Healthy patients	14	Open label <ul style="list-style-type: none"> AZD4831 AZD4831 and Midazolam UK	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: Q4 2021 Data anticipated: H1 2022
Phase I NCT04949438	Renal Impairment	20	Open label <ul style="list-style-type: none"> AZD4831 	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: H2 2022

eplontersen (ligand-conjugated antisense)

ATTR

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints (primary)	Status
Phase III CARDIO-TTRransform NCT04136171 Partnered with Ionis Pharmaceuticals Inc	Patients with hereditary or wild-type Transthyretin-Mediated Amyloid Cardiomyopathy (ATTR CM)	750	<ul style="list-style-type: none"> Arm 1: eplontersen s.c. Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: composite Outcome of Cardiovascular (CV) Mortality and recurrent CV clinical events at Week 120 Secondary endpoints include: 6MWT, KCCQ, CV Events, CV Mortality 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: 2023+
Phase III NEURO-TTRransform NCT04136184 Partnered with Ionis Pharmaceuticals Inc	Patients with hereditary transthyretin-mediated amyloid polyneuropathy (hATTR-PN)	168	<ul style="list-style-type: none"> Arm 1: eplontersen s.c. Arm 2: inotersen s.c. 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in mNIS+7 at Week 66 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: H1 2022

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



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

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III KALOS NCT04609878	Severe asthma	2200	Randomised, double-blind, double dummy, parallel group and multicentre Treatments (24 to 52 week variable length) <ul style="list-style-type: none"> BGF 320/28.8/9.6µg BID MDI BGF 320/14.4/9.6µg BID MDI BFF 320/9.6µg BID MDI Symbicort 320/9µg BID pMDI Multi-country	<ul style="list-style-type: none"> Primary endpoint: Change from baseline in forced expiratory volume in 1 second (FEV1) area under the curve 0 to 3 hours (AUC0-3) at Week 24 Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase III LOGOS NCT04609904	Severe asthma	2200	Randomised, double-blind, double dummy, parallel group and multicentre Treatments (24 to 52 week variable length) <ul style="list-style-type: none"> BGF 320/28.8/9.6µg BID MDI BGF 320/14.4/9.6µg BID MDI BFF 320/9.6µg BID MDI Symbicort 320/9µg BID pMDI Multi-country	<ul style="list-style-type: none"> Primary endpoint: Change from baseline in forced expiratory volume in 1 second (FEV1) area under the curve 0 to 3 hours (AUC0-3) at Week 24 Primary endpoint of pooled trials D5982C00007 and D5982C00008: Rate of severe asthma exacerbations Secondary endpoint: Change from baseline in morning pre-dose trough FEV1 at Week 24 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase III VATHOS NCT05202262	Moderate asthma	630	Randomized, double-blind, parallel group and multicentre Treatments (24 week) <ul style="list-style-type: none"> BFF 320/9.6µg BID MDI BFF 160/9.6µg BID MDI BD 320µg BID MDI Open-label Symbicort TBH 320/9µg BID Multi-country	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated: 2023+



Daliresp/ Daxas (PDE4 inhibitor, oral)

COPD

- Approved medicines
- Late-stage development
- Early development

Trial	Population	Patients	Design	Endpoints	Status
Post Launch PASS NCT03381573	COPD	124080	A retrospective cohort trial comparing COPD patients aged 40 years and older with new exposure to roflumilast with up to 5 unexposed (i.e., not roflumilast-exposed) COPD controls matched by propensity score (PS), age, sex, and year of cohort entry. The trial is using electronic healthcare databases in the US (Military Health System database), Germany (German Pharmacoepidemiological Research Database), Sweden (national databases including healthcare, death, and demographics data) and Norway.	<ul style="list-style-type: none">Primary endpoint: all-cause mortality (up to five years)	<ul style="list-style-type: none">Data anticipated: H2 2022



Fasenra (IL-5R mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELTEMI NCT02808819	A multicentre, open-label, safety extension trial with <i>Fasenra</i> for asthmatic adults on ICS plus LABA2 Agonist Age 18-75 years	447	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. Arm 2: <i>Fasenra</i> 30mg Q8W s.c. Global trial - 15 countries	<ul style="list-style-type: none"> Primary endpoint: safety and tolerability 	<ul style="list-style-type: none"> 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	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. 38-week trial Global trial – 16 countries	<ul style="list-style-type: none"> Primary endpoint: reduction of oral corticosteroid dose 	<ul style="list-style-type: none"> FPCD: Q3 2018 LPCD: Q3 2019 Data readout: Q4 2020 Primary endpoint met
Phase III D3250C00036 China ICS/LABA Trial (MIRACLE) NCT03186209	Severe, uncontrolled asthma, despite background controller medication, MD & HD ICS + LABA ± chronic OCS Age 12-75 years	666	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo s.c. 56-week trial Global trial – 4 countries	<ul style="list-style-type: none"> Primary endpoint: annual asthma exacerbation rate Secondary endpoints: assess pulmonary function, asthma symptoms, other asthma control metrics 	<ul style="list-style-type: none"> FPCD: Q4 2017 Data anticipated: 2023



Fasenra (IL-5R 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	2133	Randomised double-blind safety extension trial <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. Arm 2: <i>Fasenra</i> 30mg Q8W s.c. 56-week (adults) 108-week (adolescents) Global trial – 24 countries	<ul style="list-style-type: none"> Primary endpoint: safety and tolerability 	<ul style="list-style-type: none"> 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	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. 28-week (adults) Global trial – two countries	<ul style="list-style-type: none"> Primary endpoint: functionality, reliability, and performance of a pre-filled syringe with <i>Fasenra</i> administered at home 	<ul style="list-style-type: none"> FPCD: Q2 2015 Data readout: Q2 2016 Primary endpoint met
Phase III ARIA NCT02821416	Allergen-induced inflammation in mild, atopic asthma Age 18-65 years	46	A double-blind, randomised, parallel group, placebo-controlled multicentre trial to evaluate the effect of <i>Fasenra</i> on allergen-induced inflammation in mild, atopic asthma patients <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. Arm 2: placebo Q4W s.c. 37-week trial	<ul style="list-style-type: none"> Primary endpoint: safety and tolerability Primary endpoint: the effect of <i>Fasenra</i> on allergen induced eosinophil changes in sputum and allergen-induced late asthmatic response 	<ul style="list-style-type: none"> FPCD Q4 2016 LPCD: Q2 2019 Data readout: Q4 2020 Primary endpoint met
Phase III ALIZE NCT02814643	Adolescent and young adult patients with severe asthma receiving a seasonal influenza vaccine Ages 12-21 years	103	A multicentre, randomised, double-blind, parallel group, placebo-controlled trial <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. with one dose of seasonal influenza virus vaccine i.m. Arm 2: placebo Q4W s.c. with one dose of seasonal influenza virus vaccine i.m. 12-week trial	<ul style="list-style-type: none"> 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 titre 	<ul style="list-style-type: none"> FPCD: Q3 2016 Data readout: Q3 2017 Primary endpoint met



Fasenra (IL-5R mAb)

Severe, uncontrolled asthma, COPD and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III GRECO NCT02918071	Severe asthma on ICS/LABA Age 18-75 years	121	Open label • <i>Fasenra</i> 30mg Q4W 28-week trial Global trial - two countries	<ul style="list-style-type: none"> Primary endpoint: percentage of patients/caregivers who successfully self administer at home 	<ul style="list-style-type: none"> FPCD: Q4 2016 Data readout: Q4 2017 Primary endpoint met
Phase IIIb ANDHI NCT03170271	Patients with severe asthma uncontrolled on SoC treatment. Age 18-75	659	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo Q8W s.c. 24-week trial Global trial – 15 countries	<ul style="list-style-type: none"> Primary endpoint: rate of asthma exacerbations Secondary outcome measures: Saint George Respiratory Questionnaire (SGRQ) 	<ul style="list-style-type: none"> FPCD: Q3 2017 LPCD: Q1 2019 Data readout: Q4 2019 Primary endpoint met
Phase III RESOLUTE NCT04053634	Patients with moderate to very severe COPD with a history of frequent exacerbations on a background triple therapy (ICS/LABA/LAMA) Age 40-85 years	868	Double-blind, placebo controlled <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 100mg Q8W s.c. Arm 2: placebo Q8W s.c. 56-week treatment Global trial – 26 countries	<ul style="list-style-type: none"> Primary endpoint: annualized rate of moderate or severe exacerbations over 56 weeks 	<ul style="list-style-type: none"> FPCD Q4 2019 Data anticipated: 2023+
Phase III MAHALE NCT05006573	Patients With non-cystic fibrosis bronchiectasis (NCFB) with eosinophilic inflammation Age 18 years and older	420	Double blind treatment period and open label extension trial <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q4W s.c. Arm 2: placebo Q4W s.c. 52-week Global trial – 17 countries	<ul style="list-style-type: none"> Primary endpoint: annualised bronchiectasis exacerbation rate at week 52 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+
Phase I AMES NCT02968914	Healthy volunteers age 18-55 years	180	Open label trial <ul style="list-style-type: none"> <i>Fasenra</i> 30 mg PK administered by APFS device <i>Fasenra</i> 30 mg PK administered by AI device 8-week trial Global trial – two countries	<ul style="list-style-type: none"> Primary endpoint: PK comparability 	<ul style="list-style-type: none"> FPCD: Q1 2017 Data readout: Q3 2017



Fasenra (IL-5R mAb)

Nasal polyposis and other eosinophilic diseases

Trial	Population	Patients	Design	Endpoints	Status
Phase III OSTRO NCT03401229	Patients with severe bilateral nasal polyps who are still symptomatic despite standard of care therapy Age 18-75 years	413	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo s.c. 56-week trial Global trial- 8 countries	<ul style="list-style-type: none"> Primary endpoint: effect of <i>Fasenra</i> on nasal polyp burden and on patient reported nasal blockage 	<ul style="list-style-type: none"> FPCD: Q1 2018 LPCD: Q2 2019 Data readout: Q3 2020 Co-primary endpoints met
Phase III ORCHID NCT04157335	Patients with eosinophilic chronic rhinosinusitis with severe nasal polyposis Age 18-75 years	148	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 30mg Q8W s.c. Arm 2: placebo Q8W s.c. 56-week trial Global trial - 10 countries	<ul style="list-style-type: none"> Primary endpoint: Change in endoscopic total nasal polyp score and change in mean nasal blockage score 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: 2023
Phase III MANDARA NCT04157348	Patients with relapsing or refractory EGPA on corticosteroid therapy with or without stable immunosuppressive therapy Age 18 years and older	140	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 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	<ul style="list-style-type: none"> Primary endpoint: Proportion of patients achieving remission (BVAS=0 and OCS dose ≤ 4mg/day) at both weeks 36 and 48. 	<ul style="list-style-type: none"> FPCD: Q4 2019 Data anticipated: 2023
Phase III NATRON NCT04191304	Patients with HES (history of persistent eosinophilia >1500 cells/ μ L 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	<ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> 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	<ul style="list-style-type: none"> Primary endpoint: Time to first HES worsening/flare 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: 2H2022



Fasenra (IL-5R mAb)

Gastrointestinal diseases

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III MESSINA NCT04543409	Documented diagnosis of EoE Age 12 to 65 years	170	Double blind treatment period and open label period(s) • Arm 1: <i>Fasenra</i> 30mg Q4W s.c. • Arm 2: placebo Q4W s.c. 24-week Global trial – 12 countries	<ul style="list-style-type: none"> Primary endpoints: histologic response at week 24, change from baseline in DSQ score at week 24 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022
Phase III HUDSON	Patients with eosinophilic gastritis and/or gastroenteritis. Age >=12yrs	220	Double blind treatment period and open label extension • Arm 1: <i>Fasenra</i> s.c. • Arm 2: placebo s.c. 24-week Global trial	<ul style="list-style-type: none"> Dual primary endpoints at week 24: Proportion of patients achieving a histological response in the stomach and/or in the duodenum Absolute change in symptoms of EG/EGE 	<ul style="list-style-type: none"> Initiating Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Fasenra (IL-5R mAb)

Dermatology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III FJORD NCT04612790	Patients with symptomatic (newly diagnosed or relapsing) bullous pemphigoid	120	Double blind treatment period and open label period <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> Arm 2: placebo 36-week Global trial	<ul style="list-style-type: none"> Primary endpoint: Proportion of patients with complete sustained (≥ 2 months) remission off OCS at 36 weeks 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: 2023
Phase II ARROYO NCT04612725	Patients with moderate/severe chronic spontaneous urticaria, and resistant to H1 treatment	160	Double blind treatment period and open label period <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> regimen 1 Arm 2: <i>Fasenra</i> regimen 2 Arm 3: placebo 24-week Global trial	<ul style="list-style-type: none"> Primary endpoint: Change from baseline in ISS7 at week 12 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022
Phase II HILLIER NCT04605094	Patients with moderate to severe atopic dermatitis despite treatment with topical medications	160-200	Double blind treatment period and open label periods <ul style="list-style-type: none"> Arm 1: <i>Fasenra</i> Arm 2: placebo 16-week Global trial	<ul style="list-style-type: none"> Primary endpoint: Proportion of patients with an IGA 0/1 and a decrease in IGA of ≥ 2 points at week 16 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Tezspire (TSLP mAb)

Severe, uncontrolled asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III NAVIGATOR NCT03347279 Partnered	Severe asthma Age 12-80 years	1061	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52 week trial Global trial – 18 countries	<ul style="list-style-type: none"> 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) 	<ul style="list-style-type: none"> 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	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 48 week trial Global trial – seven countries	<ul style="list-style-type: none"> Primary endpoint: Reduction from baseline in daily OCS dose while not losing asthma control Secondary endpoint: Annual asthma exacerbation rate 	<ul style="list-style-type: none"> 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	951	Extension trial to NAVIGATOR and SOURCE <ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52 week trial (subjects from NAVIGATOR); 56 week trial (subjects from SOURCE) Global trial – 18 countries	<ul style="list-style-type: none"> Primary endpoint: Exposure adjusted rates of AEs/SAEs Secondary endpoints: Annual asthma exacerbation rate 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q4 2020 Data anticipated: H2 2022
Phase III PATH-HOME NCT03968978 Partnered	Severe asthma Age 12-80 years	216	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. via AI Arm 2: <i>Tezspire</i> s.c. via APFS 24 week trial Global trial – 4 countries	<ul style="list-style-type: none"> Primary endpoint: Proportion of health care professionals and patients /caregivers who successfully administered <i>Tezspire</i> in clinic and at home with an APFS or an AI, respectively 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q3 2019 Data readout: Q4 2020 Primary endpoint met



Tezspire (TSLP mAb)

Severe, uncontrolled asthma, COPD & CRSwNP

Trial	Population	Patients	Design	Endpoints	Status
Phase III WAYPOINT NCT04851964 Partnered	Severe chronic rhinosinusitis with nasal polyps (CRSwNP) Age 18+	400	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52 week trial Global trial – 11 countries	<ul style="list-style-type: none"> Co-primary endpoint: nasal polyp score and participant reported nasal congestion 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: 2023+
Phase III DIRECTION NCT03927157 Partnered	Severe asthma Age 18-80 years	396	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52 week trial Regional Asia trial – three countries	<ul style="list-style-type: none"> 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) 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: 2023+
Phase III NOZOMI NCT04048343 Partnered	Severe asthma 12-80 years	65	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. 52 week trial Local trial - Japan	<ul style="list-style-type: none"> Primary endpoint: number of patients with adverse events 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q4 2019 Data readout: Q2 2021 Primary endpoint met
Phase II CASCADE NCT03688074 Partnered	Severe asthma Age 18-75 years	116	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 28 week trial Global trial – five countries	<ul style="list-style-type: none"> Primary endpoint: number of airway submucosal inflammatory cells/mm2 of bronchoscopic biopsies 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q4 2019 Data readout: Q2 2021 Primary endpoint met
Phase IIa COURSE NCT04039113 Partnered	Moderate to very severe COPD Age 40-80	338	<ul style="list-style-type: none"> Arm 1: <i>Tezspire</i> s.c. Arm 2: placebo s.c. 52 week trial Global trial – 10 countries	<ul style="list-style-type: none"> Primary endpoint: rate of moderate or severe COPD exacerbations 	<ul style="list-style-type: none"> FPCD: Q3 2019 Data anticipated: 2023



PT027 (SABA/ICS, pMDI)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase III MANDALA NCT03769090 Managed by Avillion	Moderate to severe asthma	3123	Randomised, double-blind, multicentre, parallel group Treatments (minimum 24-week treatment period) <ul style="list-style-type: none"> PT027 (albuterol/budesonide) pMDI 180/80 µg prn PT027 (albuterol/budesonide) pMDI 180/160 µg prn AS (albuterol sulphate) pMDI 180 µg prn Multi-country	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2021 Data readout: Q3 2021 Primary endpoint met
Phase III DENALI NCT03847896 Managed by Avillion	Mild to moderate asthma	1001	Randomised, double-blind, multicentre and parallel-group Treatments (12 week treatment period) <ul style="list-style-type: none"> PT027 (albuterol/budesonide) pMDI 180/80 µg QID PT027 (albuterol/budesonide) pMDI 180/160 µg QID BD pMDI 160 µg QID AS pMDI 180 µg QID placebo pMDI QID Multi-country	<ul style="list-style-type: none"> Dual primary endpoints: Change from baseline in FEV1 AUC 0-6 hours over 12 weeks; change from baseline in trough FEV1 at week 12 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q2 2021 Data readout: Q3 2021 Dual primary endpoints met
Phase III TYREE NCT04234464 Managed by Avillion	Asthma with exercise induced bronchoconstriction	60	Randomised, double-blind, multicentre crossover Treatments (single dose) <ul style="list-style-type: none"> PT027 (albuterol/budesonide) pMDI 180/80 µg placebo pMDI QID US	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q3 2020 Data readout: Q4 2020 Primary endpoint met



Saphnelo (type I interferon receptor mAb)

Lupus (SLE / LN)

Trial	Population	Patients	Design	Endpoints	Status
Phase III TULIP-1 SLE NCT02446912	Moderate to severe SLE	450	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> Primary endpoint: SRI4 at week 52 	<ul style="list-style-type: none"> FPCD: Q4 2015 LPCD: Q4 2017 Data readout: Q3 2018 Primary endpoint not met
Phase III TULIP-2 SLE NCT02446899	Moderate to severe SLE	360	<ul style="list-style-type: none"> Arm 1: 300mg i.v. anifrolumab Q4W for 48 weeks Arm 2: placebo i.v. Q4W for 48 weeks 	<ul style="list-style-type: none"> Primary endpoint: BICLA at week 52 	<ul style="list-style-type: none"> FPCD: Q4 2015 LPCD: Q4 2017 Data readout: Q3 2019 Primary endpoint met
Phase III TULIP LTE NCT02794285	Moderate to severe SLE	630	<ul style="list-style-type: none"> Arm 1: 300mg i.v. anifrolumab Q4W for 152 weeks Arm 2: placebo i.v. Q4W for 152 weeks 	<ul style="list-style-type: none"> Primary endpoint: extension to evaluate long-term safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q2 2016 LPCD: Q4 2018 Data anticipated: H1 2022
Phase II TULIP-LN1 NCT02547922	Active proliferative LN	150	<ul style="list-style-type: none"> 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 	<ul style="list-style-type: none"> Response in proteinuria at week 52 	<ul style="list-style-type: none"> FPCD: Q4 2015 LPCD: Q4 2018 Primary endpoint not met
Phase III TULIP-SC NCT04877691	Moderate to severe SLE patients	360	<ul style="list-style-type: none"> Arm 1: <i>Saphnelo</i> s.c. Arm 2: placebo Global	<ul style="list-style-type: none"> Primary endpoint: BICLA at week 52 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+
Phase III AZALEA-SLE NCT04931563	Moderate to severe SLE patients	328	<ul style="list-style-type: none"> Arm 1: 300 mg <i>Saphnelo</i> i.v. Q4W Arm 2: 300 mg placebo i.v. Q4W Asia	<ul style="list-style-type: none"> Primary endpoint: BICLA at week 52 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+



Brazikumab (IL-23 inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb / III INTREPID NCT03759288	Crohn's disease	928	Stage 1 <ul style="list-style-type: none"> • Arm 1: brazikumab high i.v. dose on day 1, 29 and 57 + s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 2: brazikumab low i.v. dose on day 1, 29 and 57 s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 3: placebo Stage 2 <ul style="list-style-type: none"> • Arm 1: brazikumab high i.v. dose on day 1, 29 and 57 + s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 2: brazikumab low i.v. dose on day 1, 29 and 57 s.c. brazikumab on day 85 and every 4 weeks through week 48 • Arm 3: adalimumab s.c. on day 1, 15, 29 and every 2 weeks through week 50 	<ul style="list-style-type: none"> • Stage 1 primary endpoint: percentage of patients with CDAI remission at week 12 • Stage 1 secondary endpoints: Percentage of patients with endoscopic response at week 12, Percentage of patients with clinical remission at week 12 • Stage 2 primary endpoints: Percentage of patients with endoscopic response at week 52; Percentage of patients with clinical remission at week 52 • Stage 2 secondary endpoints: percentage of patients with endoscopic response at both Week 12 and Week 52; percentage of patients with clinical remission at both Week 12 and Week 52 	<ul style="list-style-type: none"> • FPCD: Q4 2018 • Data anticipated: 2023+
Phase III NCT03961815	Crohn's Disease	161	Open label extension	<ul style="list-style-type: none"> • Primary endpoint: safety of long-term treatment with brazikumab 	<ul style="list-style-type: none"> • FPCD: Q2 2019 • Data anticipated: 2023+
Phase II EXPEDITION NCT03616821	Ulcerative colitis	256	<ul style="list-style-type: none"> • Arm 1: brazikumab dose 1 i.v. on day 1, 15 and 43 + s.c. brazikumab from day 71 and every 4 weeks • Arm 2: brazikumab dose 2 i.v. on day 1, 15 and 43 + s.c. brazikumab from day 71 and every 4 weeks • Arm 3: placebo 	<ul style="list-style-type: none"> • Primary endpoint: clinical remission at week 10 • Secondary endpoint: sustained clinical remission at week 10 and 54 	<ul style="list-style-type: none"> • FPCD: Q3 2018 • Data anticipated: 2023+
Phase II NCT04277546	Ulcerative colitis	165	Open label extension	<ul style="list-style-type: none"> • Clinically significant adverse events 	<ul style="list-style-type: none"> • FPCD: Q1 2020 • Data anticipated: 2023+
Phase I NCT05033431	Healthy volunteers	48	Open-label Study to Evaluate the Pharmacokinetics, Safety and Tolerability of a Single Dose of Brazikumab Administered by IV Infusion and SC Injection in Healthy Chinese and White Participants	<ul style="list-style-type: none"> • Primary endpoints to be evaluated in healthy Chinese and White participants: • Cmax of brazikumab • AUCinf of brazikumab • AUClast of brazikumab • AUC0-28d of brazikumab 	<ul style="list-style-type: none"> • FPCD: Q4 2021 • Data anticipated: H2 2022



Tozorakimab (IL-33 ligand mAb)

COPD

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04631016	Adult subjects with COPD and chronic bronchitis	144	Randomised, double-blind, placebo-controlled, parallel group, proof of concept trial <ul style="list-style-type: none">• Arm 1: Tozorakimab s.c.• Arm 2: placebo s.c. US, Australia, Canada, Czech Republic, Denmark, Germany, Hungary, Israel, Netherlands, New Zealand, Poland, South Africa, Spain, Taiwan and UK	<ul style="list-style-type: none">• Primary endpoint: change from baseline at week 12 in FEV1• Secondary endpoints: safety and other efficacy measures	<ul style="list-style-type: none">• FPCD: Q1 2021• Data anticipated: 2023

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Tozorakimab (IL-33 ligand mAb)

Atopic dermatitis, asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04212169	Adult subjects with atopic dermatitis	152	Randomised, double-blind, placebo-controlled trial <ul style="list-style-type: none"> • Arm 1: tozorakimab s.c. • Arm 2: tozorakimab s.c. • Arm 3: tozorakimab s.c. • Arm 4: placebo s.c. US, Australia, Germany, Poland, UK & Spain	<ul style="list-style-type: none"> • Primary endpoint: change from baseline at week 16 in Eczema Area and Severity Index (EASI) score • Secondary endpoints: safety and other efficacy measures 	<ul style="list-style-type: none"> • FPCD: Q4 2019 • Data anticipated: 2023
Phase II NCT04570657	Adult participants with uncontrolled moderate to severe asthma	228	Randomised, double-blind, placebo-controlled trial <ul style="list-style-type: none"> • Arm 1: tozorakimab Dose 1 s.c. • Arm 2: tozorakimab Dose 2 s.c. • Arm 3: placebo s.c. US, Argentina, Germany, Hungary, Poland, South Africa and UK	<ul style="list-style-type: none"> • Primary endpoint: change from baseline at week 16 in FEV1 • Secondary endpoints: safety and other efficacy measures 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • Data anticipated: H2 2022
Phase I NCT05070312	Healthy subjects	36	Randomized, double-blind, placebo-controlled, doseascending trial <ul style="list-style-type: none"> • Arm 1: tozorakimab Dose 1 s.c. • Arm 2: placebo s.c. • Arm 3: tozorakimab Dose 2 s.c. • Arm 4: placebo s.c. China	<ul style="list-style-type: none"> • Primary endpoint: to characterize the pharmacokinetics of tozorakimab • Secondary endpoints: to evaluate the immunogenicity of tozorakimab 	<ul style="list-style-type: none"> • FPCD: Q3 2021 • Data anticipated: 2023



Nirsevimab (Respiratory syncytial virus mAb-YTE)

Infection

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III MELODY NCT03979313	Healthy infants (born 35 weeks 0 days or greater GA)	3000 (total) 1500 (efficacy cohort) 1500 (safety cohort)	Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. Global trial – 31 countries	<ul style="list-style-type: none"> Primary endpoints: efficacy Secondary endpoints: safety, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q3 2019 (efficacy cohort) LPCD: Q1 2020 (efficacy cohort) Data readout: Q2 2021 (efficacy cohort) Primary endpoint met FPCD: Q2 2021 (safety cohort) LPCD: Q4 2021 (safety cohort) Data anticipated: H2 2022 (safety cohort)
Phase III CHIMES NCT05110261	Healthy infants (born 29 weeks 0 days or greater GA)	800	Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab i.m. Arm 2: placebo i.m. China Only	<ul style="list-style-type: none"> Primary endpoints: efficacy Secondary endpoints: safety, PK, ADA 	<ul style="list-style-type: none"> FPCD: Q4 2021 LPCD: 2023+ Data anticipated: 2023+
Phase II/III MEDLEY NCT03959488	High risk preterm (born 35 weeks 0 day or less GA), CHD and CLD infants eligible to receive <i>Synagis</i>	925	Randomised, Double-blind, palivizumab-controlled Arm 1: nirsevimab i.m. Arm 2: <i>Synagis</i> i.m. Global trial – 32 countries	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, ADA and descriptive efficacy 	<ul style="list-style-type: none"> FPCD: Q3 2019 LPCD: Q4 2021 Data readout: Q2 2021 Safety objective met
Phase IIb NCT02878330	29-35 WK GA (gestational age) infants	1453	Randomised, double-blind, placebo-controlled trial Arm 1: nirsevimab i.m. Arm 2: placebo i.m.	<ul style="list-style-type: none"> Primary endpoints: safety and efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2016 LPCD: Q4 2017 Data readout: Q4 2018 Primary endpoint met
Phase II MUSIC NCT04484935	Immunocompromised children who are ≤ 24 months of age at the time of dose administration	100	Open-label, Uncontrolled, single-dose trial • nirsevimab i.m. Route of administration: i.m. Global trial – 8 countries	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, ADA, efficacy 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: 2023
Phase I China NCT04840849	Healthy Chinese adults, 18-45 years of age	24	Randomised, Double-blind, placebo-controlled Arm 1: nirsevimab -i.m. Arm 2: placebo i.m. Route of administration: i.m. China only	<ul style="list-style-type: none"> Primary endpoint: PK Secondary endpoints: ADA, safety 	<ul style="list-style-type: none"> FPCD: Q2 2021 LPCD: Q2 2021 Data anticipated: H1 2022



AZD1222/AZD2816 (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
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	10812	Single-blinded, randomised, controlled, multicentre trial with sequential age escalation/de-escalation immunogenicity sub-studies that include prime boost • AZD1222 • Control vaccine: MenACWY UK	<ul style="list-style-type: none"> Primary endpoint: efficacy and safety Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q4 2020
Phase III D8110C00001 (US, global) NCT04516746	Healthy adults Aged 18-65 years	32429	Adaptive, double-blinded, randomised placebo-controlled trial • AZD1222 • placebo US, Peru, Chile	<ul style="list-style-type: none"> Primary endpoints: efficacy, safety, tolerability, and reactogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q1 2021 Primary 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	10416	Single-blinded, randomised, controlled multicentre trial • AZD1222 • Control vaccine: MenACWY Brazil	<ul style="list-style-type: none"> Primary endpoint: efficacy Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q4 2020
Phase II/III AZD2816 (UK, Brazil, South Africa, Poland) NCT04973449	Healthy adults Aged > 18 years	2590	Partially double-blinded, randomized, active-controlled trial in unvaccinated or previously vaccinated participants • AZD2816 • AZD1222	<ul style="list-style-type: none"> Primary endpoint: safety, tolerability Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data readout Q1 2022 Primary endpoint met
Phase I/II COV001 (UK) NCT04324606 Partnered	Healthy adults Age 18-55 years	1077	Single-blinded, randomised, controlled, multicentre trial • AZD1222 • Control vaccine: MenACWY UK	<ul style="list-style-type: none"> Primary endpoint: efficacy and safety Secondary endpoints: safety, tolerability, reactogenicity, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q2 2020
Phase I/II COV005 (SA) NCT04444674 Partnered	Healthy adults Age 18-65 years HIV+ subgroup	2130	Adaptive, double-blinded, randomised placebo-controlled trial • AZD1222 • placebo South Africa	<ul style="list-style-type: none"> Primary endpoint: efficacy, safety, and immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2020 LPCD: Q4 2020



AZD1222/AZD2816 (SARS-CoV-2)

Prevention of COVID-19

Trial	Population	Patients	Design	Endpoints	Status
Phase I/II D8111C00002 (Japan) NCT04568031	Healthy adults Age ≥18 years	256	Double-blinded, randomised, placebo-controlled multicentre trial • AZD1222 • placebo Japan	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability, reactogenicity, immunogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2020 Data readout: Q4 2020
Phase I/II COV004 (Kenya) Partnered	Healthy adults	400	Double-blinded, randomised, placebo-controlled multicentre trial • AZD1222 • Control vaccine: rabies Kenya	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability, reactogenicity, immunogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2021
Phase II COV006 (UK) ISRCTN15638344 Partnered	Healthy children and adolescents age 6-17 years	261	Single-blinded, randomized multicentre trial • AZD1222 • Meningococcal Group B vaccine UK	<ul style="list-style-type: none"> Primary endpoints: safety, tolerability, reactogenicity Secondary endpoints: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q2 2021
Phase I COV008 (UK) NCT04816019 Partnered	Healthy adults Age 18-55	54	Open label, randomized, dose-escalation trial of AZD1222 administered intranasally • AZD1222 UK	<ul style="list-style-type: none"> Primary endpoint: safety, tolerability Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q4 2020



Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients/Subjects	Design	Endpoints	Status
Phase III PROVENT D8850C00002 NCT04625725	Adults having increased risk for inadequate response to active immunisation or having increased risk for SARS-CoV-2 infection	5197	Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in pre-exposure prophylaxis <ul style="list-style-type: none"> Arm 1: <i>Evusheld</i> Arm 2: placebo <i>Evusheld</i> /placebo (2:1) USA, UK, Belgium, France, Spain	<ul style="list-style-type: none"> Primary endpoint: positive symptomatic illness post –dose Secondary endpoints: Incidence of: nucleocapsid antibodies, emergency visits, PCR positive, ADA to <i>Evusheld</i> in serum and <i>Evusheld</i> serum conc. 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q1 2021 Data readout: Q3 2021 Primary endpoint met
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	1121	Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy in post-exposure prophylaxis <ul style="list-style-type: none"> Arm 1: <i>Evusheld</i> Arm 2: placebo <i>Evusheld</i> /placebo (2:1) USA and UK	<ul style="list-style-type: none"> Primary endpoint: positive symptomatic illness post –dose Secondary endpoints: Incidence of: nucleocapsid antibodies, COVID-19 related death, all cause mortality, ADA to <i>Evusheld</i> in serum and AZD7442 serum conc. 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q1 2021 Data readout: Q2 2021 Primary endpoint not met
PHASE III TACKLE NCT04723394	Adults with confirmed mild to moderate SARS-COV2 infection. Symptomatic patients with documented positive SARS-Cov-2 molecular test	910	Double-blinded, randomised, placebo controlled, multi centre study to determine safety and efficacy for treatment of Covid-19 in non-hospitalised patients <ul style="list-style-type: none"> Arm 1: <i>Evusheld</i> Arm 2: placebo <i>Evusheld</i> /placebo (1:1) UK, Germany, Spain, Italy, Hungary, Russia, US, Mexico, Japan, Poland, Czech Republic, Argentina, Brazil, and Ukraine	<ul style="list-style-type: none"> Primary endpoint: efficacy in the prevention of the composite endpoint of either severe COVID-19 or death from any cause through study day 29 Secondary endpoints: A composite of either death from any cause or hospitalisation for COVID-19 complications or sequelae (Day 1 to Day 169). Determine symptom severity and prevention of respiratory failure 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q3 2021 Data readout: Q4 2021 Primary endpoint met



Evusheld (AZD7442, tixagevimab + cilgavimab)

Prevention and treatment of COVID-19

Trial	Population	Patients/Subjects	Design	Endpoints	Status
Phase I NCT04507256	Healthy adults Aged 18-55 years	60	Double-blinded, randomised, placebo controlled, single ascending dose trial <ul style="list-style-type: none"> Arm 1: <i>Evusheld</i> Arm 2: placebo <i>Evusheld</i> /placebo (10:2) UK	<ul style="list-style-type: none"> Primary endpoint: safety, tolerability and PK Secondary endpoint: immunogenicity 	<ul style="list-style-type: none"> FPPD: Q3 2020 LPCD: Q3 2020 Data readout: Q1 2022
Phase I PK Co-formulation NCT05166421	Healthy adults Aged ≥ 18 years	207	Open-label, randomised three-arm, single dose trial <ul style="list-style-type: none"> Arm 1: <i>Evusheld</i> administered as a single co-formulated dose (clonal cell line material) Arm 2: <i>Evusheld</i> administered as two separate doses (clonal cell line material) Arm 3: <i>Evusheld</i> administered as two separate doses (cell pool material) <i>Evusheld</i> (1:1:1) USA	<ul style="list-style-type: none"> Key Endpoints: safety and PK 	<ul style="list-style-type: none"> FPCD: Q1 2022 Data anticipated H2 2022



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	<ul style="list-style-type: none"> Part A: cotadutide or placebo s.c. Part B: cotadutide s.c. or placebo s.c. or liraglutide s.c. Sweden, Netherlands, UK	<ul style="list-style-type: none"> Primary endpoint: change in hepatic glycogen concentration postprandially, adjusted by liver volume Secondary endpoints: safety, tolerability, immunogenicity 	<ul style="list-style-type: none"> FPCD: Q2 2018 Part A LPCD: Q4 2018 Data readout: Q1 2019 Part B FPCD: Q1 2020 LPCD: Q1 2021 Data readout: Q2 2021
Phase IIb NCT03596177	Overweight and obese patients with type-2 diabetes	27	<ul style="list-style-type: none"> Cotadutide or placebo s.c. UK	<ul style="list-style-type: none"> Primary endpoints: efficacy body weight loss Secondary endpoint: change in total energy intake; change in total energy expenditure, active energy expenditure, resting energy expenditure; safety 	<ul style="list-style-type: none"> 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)	74	<ul style="list-style-type: none"> Arm 1: cotadutide high dose s.c. Arm 2: placebo high dose s.c. Arm 3: cotadutide low dose s.c. Arm 4: placebo low dose s.c. US	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: change in hepatic fat fraction; change in liver fat volume; change in visceral adipose tissue 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q2 2021 Data anticipated: H1 2022
Phase II NCT04515849	Chronic kidney disease with type 2 diabetes mellitus	225	<ul style="list-style-type: none"> Arm 1: cotadutide 100 micrograms Arm 2: cotadutide 300 micrograms Arm 3: cotadutide 600 micrograms Arm 4: semaglutide Arm 5: placebo 	<ul style="list-style-type: none"> Primary endpoint: efficacy change in UACR Secondary endpoints: Change in HbA1c; change in glucose measured by CGM; effects on body weight; safety, tolerability, Immunogenicity 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q3 2021 Data anticipated: H1 2022
Phase I NCT04091373	Healthy adult patients	36		<ul style="list-style-type: none"> Primary endpoint: exposure following a single s.c. of cotadutide at each of 3 different sites of injection Secondary endpoints: immunogenicity; safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q1 2020 Data readout: Q4 2020



AZD2373

Chronic kidney disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04269031	Healthy volunteers	30	SAD. Dose escalation in 6 cohorts with 6 volunteers receiving AZD2373 and 2 volunteers receiving placebo in each cohort <ul style="list-style-type: none">Arm 1: AZD2373 s.c.Arm 2: placebo s.c. US	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoint: PK parameters	<ul style="list-style-type: none">FPCD: Q1 2020LPCD: Q3 2021Data anticipated: H1 2022



AZD2693 (antisense oligonucleotide)

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04142424	Healthy volunteers	62	SAD. 6 cohorts with 6 volunteers receiving AZD2693 and 2 volunteers receiving placebo in each cohort <ul style="list-style-type: none">• Arm 1: AZD2693 s.c.• Arm 2: placebo s.c. US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoint: PK	<ul style="list-style-type: none">• FPCD: Q4 2019• Data readout: Q4 2021
Phase I NCT04142424	NASH/NAFLD F0-F3	60	MAD. 3 cohorts receiving AZD2693 and placebo in each cohort <ul style="list-style-type: none">• Arm 1: AZD2693 s.c.• Arm 2: placebo s.c. US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoint: PK	<ul style="list-style-type: none">• FPCD: Q2 2021• Data anticipated: H1 2022



AZD7503 (antisense oligonucleotide)

Approved medicines

Late-stage development

Early development

Oncology

NASH

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05143905	Healthy volunteers	64	SAD. 6 cohorts with 6 volunteers receiving AZD7503 and 2 volunteers receiving placebo in each cohort <ul style="list-style-type: none">• Arm 1: AZD7503 s.c.• Arm 2: placebo s.c. US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoint: PK	<ul style="list-style-type: none">• FPCD: Q4 2021• Data anticipated: 2023

CVRM

R&I

Other

COVID-19

Rare Disease



AZD3427 (relaxin)

Heart failure

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04630067	SAD – Healthy volunteers MAD – Heart failure	104	Multicentre single and multiple ascending dose study Part A SAD 6 cohorts • Arm 1: AZD3427 • Arm 2: placebo Part B MAD • Arm 1: AZD3427 • Arm 2: placebo US	<ul style="list-style-type: none">Primary endpoints: safety and tolerability	<ul style="list-style-type: none">FPCD: Q4 2020Data anticipated: H2 2022



AZD3366

Cardiovascular disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04588727	Healthy volunteers	103	<p>SAD trial Part A 7 cohorts with 6 volunteers receiving AZD3366 and 2 volunteers receiving placebo in each cohort; three cohorts with Japanese subjects 5 receiving AZD3366 1 receiving placebo; 1 Chinese cohort of 6 receiving AZD3366 2 receiving placebo</p> <ul style="list-style-type: none">• Arm 1: AZD3366• Arm 2: placebo <p>Part B 12 subjects</p> <ul style="list-style-type: none">• Arm 1: AZD3366 + <i>Brilinta</i> + ASA• Arm 2: placebo + <i>Brilinta</i> + ASA	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoint: PK parameters	<ul style="list-style-type: none">• FPCD: Q4 2020• Data anticipated: H1 2022



AZD5718 (FLAP inhibitor)

Cardiovascular disease & Chronic Kidney Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04492722	Proteinuric CKD	632	Randomised, double-blind, placebo-controlled, multicentre, dose-ranging trial <ul style="list-style-type: none"> AZD5718 placebo 	<ul style="list-style-type: none"> Primary endpoints: dose-response efficacy, safety, PK 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023
Phase IIa NCT03317002	CAD	129	<ul style="list-style-type: none"> Arm 1: AZD5718 Dose A Arm 2: AZD5718 Dose B Arm 3: placebo Global trial – three countries in Europe	<ul style="list-style-type: none"> Primary endpoint: PD effect of AZD5718 by assessment of u-LTE4 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2019 Data readout: Q1 2021
Phase I NCT03948451	Healthy patients	6	Open label hADME trial <ul style="list-style-type: none"> a single oral dose of ¹⁴C-AZD5718 UK	<ul style="list-style-type: none"> Primary endpoint: Mass balance, with routes and rates of elimination of ¹⁴C-AZD5718; Metabolite profiling and structural identification; PK and total radioactivity 	<ul style="list-style-type: none"> FPCD: Q2 2019 LPCD: Q2 2019 Data readout: Q4 2019
Phase I NCT04087187	Healthy patients	14	BA trial. Open-label, randomised, 3-period, 3-treatment, crossover design <ul style="list-style-type: none"> AZD5718 	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability, PK 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q4 2019 Data readout: Q1 2020
Phase I NCT04210388	Healthy patients	12	BA trial. Open-label, randomised, single-dose, combined 2x2 dose and 3x3 dose crossover design in fixed sequence. <ul style="list-style-type: none"> AZD5718 	<ul style="list-style-type: none"> Primary endpoints: bioavailability, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q1 2020 Data readout: Q2 2020
Phase I NCT0473275	Healthy patients	16	BA trial. Open-label, randomised, single-dose, 3-period, single dose, crossover design <ul style="list-style-type: none"> AZD5718 	<ul style="list-style-type: none"> Primary endpoints: bioavailability, safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2021 Data readout: Q4 2021



AZD8233 (PCSK9 inhibitor, subcutaneous)

Dyslipidaemia

Trial	Population	Patients	Design	Endpoints	Status
Phase II SOLANO NCT04964557	Dyslipidaemia	376	<ul style="list-style-type: none"> Arm 1: AZD8233 dose Arm 2: placebo 28 weeks 8 countries (US, Slovakia, Denmark, Poland, Spain, Netherlands, Czech Republic, Hungary)	<ul style="list-style-type: none"> Primary endpoint: safety 	<ul style="list-style-type: none"> FPCD: Q3 2021 LPCD: Q4 2021 Data anticipated: H2 2022
Phase II ETESIAN NCT04641299	Dyslipidaemia	108	<ul style="list-style-type: none"> Arm 1: High AZD8233 dose Arm 2: Medium AZD8233 dose Arm 3: Low AZD8233 dose Arm 4: placebo 12 weeks 3 countries (US, Slovakia and Denmark)	<ul style="list-style-type: none"> Primary endpoint: efficacy 	<ul style="list-style-type: none"> FPCD: Q4 2020 LPCD: Q1 2021 Data readout: Q3 2021
Phase II HAYATE NCT04823611	Dyslipidaemia	71	Part A: 11 subjects randomized in an 8:3 ratio <ul style="list-style-type: none"> Arm 1: High AZD8233 dose Arm 2: placebo Part B: 60 subjects randomized across three different treatment arms in a 1:1:1 ratio <ul style="list-style-type: none"> Arm 1: Medium AZD8233 dose Arm 2: Low AZD8233 dose Arm 3: placebo 12 weeks Japan	<ul style="list-style-type: none"> Part A primary endpoints: safety and tolerability Part B primary endpoint: efficacy 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: H2 2022
Phase I NCT03593785	Healthy subjects	72	SAD trial 7 cohorts <ul style="list-style-type: none"> Arm1: AZD8233 Arm 2: placebo US	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK and PD parameters 	<ul style="list-style-type: none"> FPCD: Q3 2018 LPCD: Q3 2019 Data readout: Q2 2021
Phase I NCT04155645	Dyslipidaemia	33	MAD trial 3 cohorts <ul style="list-style-type: none"> Arm1: AZD8233 Arm 2: placebo US	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK and PD parameters 	<ul style="list-style-type: none"> FPCD: Q1 2020 LPCD: Q4 2020 Data readout: Q3 2021



MEDI8367

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04365218	Healthy volunteers CKD	70	SAD trial 6 cohorts <ul style="list-style-type: none">Arm 1: MEDI8367 s.c.Arm 2: placebo s.c. US	<ul style="list-style-type: none">Primary endpoints: safety and tolerabilitySecondary endpoints: PK parameters, ADA	<ul style="list-style-type: none">FPCD: Q3 2020LPCD: Q4 2020Data anticipated: H1 2022



AZD8601 (VEGF-A modified RNA)

Cardiovascular disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa NCTT03370887 EPICCURE	HF	Up to 33	<ul style="list-style-type: none">• Arm 1: AZD8601 Dose A• Arm 2: AZD8601 Dose B• Arm 3: placebo Finland, Germany	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability	<ul style="list-style-type: none">• FPCD: Q1 2018• LPCD: Q1 2021• Data readout: Q3 2021



AZD9977 (MR modulator)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04595370	Heart failure with chronic kidney disease	540	Randomised, stratified according to T2DM and eGFR (≥ 20 to < 30 mL/min / ≥ 30 to < 45 mL/min / ≥ 45 mL/min) for 12 weeks: <ul style="list-style-type: none"> Arm 1: AZD9977 A + <i>Farxiga</i> 10 mg Arm 2: AZD9977 B + <i>Farxiga</i> 10 mg Arm 3: AZD9977 C + <i>Farxiga</i> 10 mg Arm 4: AZD9977 C Arm 5: <i>Farxiga</i> 10 mg Arm 6: placebo 12 weeks Trial conducted in 20 countries globally	<ul style="list-style-type: none"> Primary endpoint: percent change from baseline in UACR at 12 weeks Secondary endpoints: percent change from baseline in UACR at 12 weeks to assess dose-response relationship; dose-response relationship of placebo, AZD9977 alone <i>Farxiga</i> and 3 doses of AZD9977 combined with <i>Farxiga</i> on UACR; safety, tolerability and serum potassium values; eGFR 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: 2023
Phase I NCT03435276	Healthy volunteers	27	MAD trial 3 cohorts <ul style="list-style-type: none"> Arm 1: AZD9977 Arm 2: placebo UK.	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2018 LPCD: Q2 2018 Data readout: Q3 2018
Phase I NCT03450759	Healthy volunteers	12	BA trial of four different oral formulations of AZD9977 and influence of food UK.	<ul style="list-style-type: none"> Primary endpoints: relative bioavailability vs. oral suspension (reference) Secondary endpoints: PK parameters 	<ul style="list-style-type: none"> FPCD: Q2 2018 LPCD: Q2 2018 Data readout: Q3 2018
Phase I NCT03682497	HF	60	<ul style="list-style-type: none"> Arm 1: AZD9977 Arm 2: spironolactone 	<ul style="list-style-type: none"> Primary endpoint: serum potassium 	<ul style="list-style-type: none"> FPCD: Q4 2018 LPCD: Q1 2019
Phase I NCT03843060	Healthy volunteers	14	DDI trial <ul style="list-style-type: none"> Arm 1: AZD9977 Arm 2: AZD9977 + itraconazole US	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q1 2019 Data readout: Q3 2019



AZD9977 (MR modulator)

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03801967	Healthy volunteers	45	JSMAD trial Single and multiple-ascending dose administration in Japanese healthy volunteers. UK	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q2 2019 Data readout: Q3 2019
Phase I NCT03804645	Healthy volunteers	12	BA trial Investigation of four different oral formulations of AZD9977 and influence of food. UK	<ul style="list-style-type: none"> Primary endpoints: relative bioavailability vs. capsule formulation (reference); PK parameters 	<ul style="list-style-type: none"> FPCD: Q1 2019 LPCD: Q2 2019 Data readout: Q3 2019
Phase I NCT04469907	Renal impairment	18	<ul style="list-style-type: none"> AZD9977 US	<ul style="list-style-type: none"> Primary endpoints: PK parameters Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q4 2021 Data anticipated: H1 2022
Phase I NCT04686591	Healthy volunteers	8	hADME trial <ul style="list-style-type: none"> single oral dose ¹⁴C-AZD9977 UK	<ul style="list-style-type: none"> Primary endpoints: absolute bioavailability, the mass balance, rates and routes of elimination Secondary endpoints: safety and tolerability 	<ul style="list-style-type: none"> FPCD: Q1 2021 LPCD: Q1 2021 Data anticipated: H1 2022
Phase I NCT04798222	Healthy volunteers	20	Rel BA trial Study to Assess the Bioavailability of Different Formulations of AZD9977 and Dapagliflozin and Influence of Food in Selected Formulations Germany	<ul style="list-style-type: none"> Primary endpoints: relative bioavailability of 9977 and Dapagliflozin in different capsule formulations; PK parameters 	<ul style="list-style-type: none"> FPCD: Q3 2021 LPCD: Q3 2021 Data anticipated: H1 2022



Zibotentan (endothelin receptor antagonist)

Chronic kidney disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb ZENITH-CKD NCT04724837	CKD	660	<p>Part A: 132 participants equally randomised across 4 arms:</p> <ul style="list-style-type: none"> Arm 1: zibotentan dose A + <i>Farxiga</i> 10 mg once daily Arm 2: zibotentan dose A once daily Arm 3: <i>Farxiga</i> 10 mg once daily Arm 4: placebo once daily <p>Part B: 528 participants equally randomised across 6 arms:</p> <ul style="list-style-type: none"> Arm 1: zibotentan dose C + <i>Farxiga</i> 10 mg once daily Arm 2: zibotentan dose B + <i>Farxiga</i> 10 mg once daily Arm 3: zibotentan dose A + <i>Farxiga</i> 10 mg once daily Arm 4: zibotentan dose A once daily Arm 5: <i>Farxiga</i> 10 mg once daily Arm 6: placebo once daily <p>Global</p>	<ul style="list-style-type: none"> 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; least squares mean change of UACR at week 12 from the 3 Zibo/Dapa dose groups and the <i>Farxiga</i> monotherapy group; change in eGFR from baseline to week 1, week 12 and week 14; change in eGFR from week 1 to week 12. 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: H2 2022
Phase 1 NCT04991571	Healthy volunteers	28	<ul style="list-style-type: none"> Part 1 of the study is intended to collect samples for Metabolites in Safety Testing (MIST) analysis after administration of multiple doses of zibotentan. Part 2 of the study is designed to evaluate the relative bioavailability of zibotentan and dapagliflozin after dosing with two different fixed-dose combination (FDC) formulations and dosing with separate formulations of zibotentan and dapagliflozin 	<p>Part 1: Metabolites in Safety Testing MIST analysis. No PK statistical analysis will be performed.</p> <p>Part 2: PK parameters</p>	<ul style="list-style-type: none"> FPCD: Q3 2021 LPCD Q4 2021 Data anticipated: H1 2022



Zibotentan (endothelin receptor antagonist)

Liver Cirrhosis with Features of portal hypertension

Trial	Population	Patients	Design	Endpoints	Status
Phase 1 NCT05112419	Cohort 1: Moderate hepatic impairment/moderate renal impairment patients Cohort 2: Healthy volunteers	12+12	Single dose, single centre, Open label, Parallel group study to investigate the pharmacokinetics, safety and tolerability of 5 mg Zibotentan	Primary Endpoints: <ul style="list-style-type: none"> Area under plasma concentration-time curve from time zero to infinity (AUCinf) Area under the plasma concentration-curve from time zero to time of last quantifiable concentration (AUClast) Maximum observed plasma concentration (Cmax) 	<ul style="list-style-type: none"> FPCD Q4 2021 LPCD Q4 2021 Data anticipated: H1 2022



AZD5462 (relaxin)

Heart failure

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04994106	SAD – Healthy volunteers MAD – Healthy volunteers		Single centre single and multiple ascending dose study Part A SAD 8 cohorts <ul style="list-style-type: none">• Arm 1: AZD5462• Arm 2: placebo Part B MAD 5 cohorts <ul style="list-style-type: none">• Arm 1: AZD5462• Arm 2: placebo US	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability	<ul style="list-style-type: none">• FPCD: Q4 2021• Data anticipated: H1 2022



MEDI6570

Cardiovascular

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04610892	Post MI	400	Evaluation of anti-inflammatory potential and effect on surrogates for atherosclerotic and heart failure (HF) events <ul style="list-style-type: none">• Arm 1: High MEDI6570 dose• Arm 2: Medium MEDI6570 dose• Arm 3: Low MEDI6570 dose• Arm 4: placebo US, Canada, Hungary, Japan, Czech Republic, Italy, Spain, Netherlands, Poland, UK, Australia, Russia	<ul style="list-style-type: none">• Primary endpoints: efficacy and safety	<ul style="list-style-type: none">• FPCD: Q4 2020• Data anticipated: 2023



Tozorakimab (IL-33 ligand mAb)

Diabetic kidney disease

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT04170543	Adult patients with diabetic kidney disease	565	<ul style="list-style-type: none">• Arm A: tozorakimab dose 1 + <i>Farxiga</i>• Arm B: tozorakimab dose 2 + <i>Farxiga</i>• Arm C: tozorakimab dose 3 + <i>Farxiga</i>• Arm D: tozorakimab dose 4 + <i>Farxiga</i>• Arm E: placebo + <i>Farxiga</i> USA, Canada, Japan and additional countries.	<ul style="list-style-type: none">• Primary endpoints: safety and efficacy	<ul style="list-style-type: none">• FPCD: Q4 2019• Data anticipated: 2023



AZD4604 (inhaled JAK-1 inhibitor)

Asthma

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04769869	Healthy subjects and patients with mild asthma	137	SAD/MAD/POM trial Part 1 SAD <ul style="list-style-type: none">• Arm 1: AZD0449 (DPI)• Arm 2: placebo (DPI) Part 2 MAD <ul style="list-style-type: none">• Arm 1: AZD0449 (DPI)• Arm 2: placebo (DPI) Part 3 POM <ul style="list-style-type: none">• Arm 1: AZD0449 (DPI)• Arm 2: placebo (DPI) UK	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: PK parameters, FENO	<ul style="list-style-type: none">• FPCD: Q4 2021• Data anticipated: 2023



AZD1402 (IL-4 receptor alpha antagonist)

Asthma

Trial	Population	Patients	Design	Endpoints	Status
Phase IIa NCT04643158	Patients with asthma on medium dose inhaled corticosteroids	345	<p>Randomised, placebo-controlled, double-blinded, multicentre, 2-part trial.</p> <p>Part 1 population with asthma controlled on medium dose ICS-LABA</p> <p>Part 1a</p> <ul style="list-style-type: none"> • Arm 1: AZD1402 Dose 1 (low) (DPI) • Arm 2: AZD1402 Dose 2 (DPI) • Arm 3: placebo <p>Part 1b</p> <ul style="list-style-type: none"> • Arm 1: AZD1402 Dose 3 (high) (DPI) • Arm 2: placebo <p>Part 2 population uncontrolled on medium dose ICS-LABA</p> <ul style="list-style-type: none"> • Arm 1: AZD1402 Dose 1 (DPI) • Arm 2: AZD1402 Dose 2 (DPI) • Arm 3: AZD1402 Dose 3 (DPI) • Arm 4: placebo <p>Ukraine, Australia, Germany, Hungary, Korea, Poland, Spain, UK</p>	<ul style="list-style-type: none"> • Part 1 primary endpoints: safety and tolerability, PK • Part 2 primary endpoint: change in FEV1 	<ul style="list-style-type: none"> • FPCD: Q2 2021 • Data anticipated: H2 2022



AZD5055 (oral porcupine inhibitor)

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

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05134727	Healthy subjects	104	SAD/MAD trial Part 1a (fasted state) and 1b (fed state) SAD <ul style="list-style-type: none"> • Arm 1: AZD5055 (oral suspension) • Arm 2: Placebo (oral suspension) Part 2 MAD <ul style="list-style-type: none"> • Arm 1: AZD5055 (oral suspension) • Arm 2: Placebo (oral suspension) 	<ul style="list-style-type: none"> • Primary endpoints: safety and tolerability • Secondary endpoints: PK parameters, food effect 	<ul style="list-style-type: none"> • FPCD: Q4 2021 • Data anticipated: 2023



AZD8630 (inhaled TSLP)

Asthma

Approved medicines

Late-stage development

Early development

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT05110976 Partnered	Healthy subjects and patients with asthma	232	SAD & MAD trial	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoints: PK parameters, FENO	<ul style="list-style-type: none">• FPCD: Q4 2021• Data anticipated: 2023



MEDI0618 (PAR2 antagonist mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT04198558	Healthy volunteers	64	SAD trial <ul style="list-style-type: none">• Arm 1: MEDI0618 i.v.• Arm 2: placebo i.v.• Arm 3: MEDI0618 s.c• Arm 4: placebo s.c Europe only	<ul style="list-style-type: none">• Primary endpoints: safety and tolerability• Secondary endpoint: PK	<ul style="list-style-type: none">• FPCD: Q4 2019• Data anticipated: H1 2022



MEDI1341 (alpha-synuclein mAb)

Parkinson's disease

Trial	Population	Patients	Design	Endpoints	Status
Phase I NCT03272165	Healthy volunteers	48	SAD trial <ul style="list-style-type: none"> Arm 1: MEDI1341 i.v. Arm 2: placebo i.v. US only	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, PD 	<ul style="list-style-type: none"> FPCD: Q4 2017 LPCD: Q4 2020 Data readout: Q4 2021
Phase I NCT04449484	Parkinson's Disease	36	MAD trial <ul style="list-style-type: none"> Arm 1: MEDI1341 i.v. Arm 2: placebo i.v. US only	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, PD 	<ul style="list-style-type: none"> FPCD: Q3 2020 LPCD: Q3 2021 Data anticipated: H1 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	Randomised, double blind, SAD trial <ul style="list-style-type: none"> • Arm 1: AZD4041 • Arm 2: placebo US only	<ul style="list-style-type: none"> • Primary endpoints: safety and tolerability • Secondary endpoints: PK, PD 	<ul style="list-style-type: none"> • FPCD: Q4 2019 • Data readout Q4 2021
Phase I NCT05209334 Partnered with Eolas Therapeutics Inc and NIH	Healthy volunteers	36	Randomised, double blind MAD trial <ul style="list-style-type: none"> • Arm 1: AZD4041 • Arm 2: placebo Canada only	<ul style="list-style-type: none"> • Primary Endpoints: safety and tolerability • Secondary endpoint: PK 	<ul style="list-style-type: none"> • FPCD: Q1 2022 • Data anticipated: H2 2022



MEDI7352 (NGF TNF bispecific mAb)

Osteoarthritis pain

Trial	Population	Patients	Design	Endpoints	Status
Phase IIb NCT04675034	Painful osteoarthritis of the knee	300	MAD trial <ul style="list-style-type: none"> Arm 1: MEDI7352 s.c Arm 2: placebo s.c Global (7 countries)	<ul style="list-style-type: none"> Primary endpoint: dose response Secondary endpoints: safety, tolerability, PK, PD, ADA 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023
Phase IIa NCT03755934	Painful diabetic neuropathy	271	MAD trial <ul style="list-style-type: none"> Arm 1: MEDI7352 i.v. Arm 2: placebo i.v. Europe only	<ul style="list-style-type: none"> Primary endpoint: dose response Secondary endpoints: safety, tolerability, PK, PD 	<ul style="list-style-type: none"> FPCD: Q4 2018 Data anticipated: 2023
Phase I NCT02508155	Painful osteoarthritis of the knee	160	SAD & MAD trial <ul style="list-style-type: none"> Arm 1: MEDI7352 i.v. Arm 2: placebo i.v. Arm 3: MEDI7352 s.c Arm 4: placebo s.c Europe only	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, PD 	<ul style="list-style-type: none"> FPCD: Q1 2016 LPCD: Q4 2020 Data readout: Q2 2021
Phase I NCT04770428	Healthy volunteers Japanese and Caucasian	20	MAD trial <ul style="list-style-type: none"> Arm 1: MEDI7352 s.c Arm 2: placebo s.c Europe only	<ul style="list-style-type: none"> Primary endpoints: safety and tolerability Secondary endpoints: PK, PD, ADA 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data readout: Q4 2021



MEDI3902 (Anti-Pseudomonas A mAb)

Infections

Trial	Population	Patients	Design	Endpoints	Status
Phase II NCT02696902	Intubated ICU	195	SAD trial <ul style="list-style-type: none">Arm 1: MEDI3902 i.v.Arm 2: placebo i.v.	<ul style="list-style-type: none">Primary endpoints: safety and efficacy	<ul style="list-style-type: none">FPCD: Q2 2016Data readout: Q4 2020



**Rare Disease – approved
medicines and late-stage
pipeline**



Soliris (anti-complement C5 mAb)

Neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ECU-GBS-301 NCT04752566	Guillain Barré syndrome	57	<ul style="list-style-type: none">• Arm 1: <i>Soliris</i> i.v. once weekly for 4 weeks• Arm 2: Placebo Japan-only	<ul style="list-style-type: none">• Primary endpoint: Time to first reaching a Hughes functional grading scale score ≤ 1	<ul style="list-style-type: none">• FPCD: Q1 2021• Data anticipated: 2023

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



Ultomiris (anti-complement C5 mAb)

Haematology & nephrology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN-1210-PNH-303 NCT03748823	PNH and aHUS	136	<ul style="list-style-type: none"> • <i>Ultomiris</i> s.c. 	<ul style="list-style-type: none"> • Primary endpoint: Day 71 serum <i>Ultomiris</i> C_{trough} 	<ul style="list-style-type: none"> • FPCD: Q1 2019 • Data readout: Q2 2020 • Primary endpoint met
Phase III ALXN-1210-TMA-315 NCT04743804	Thrombotic microangiopathy associated with a trigger	100	<ul style="list-style-type: none"> • Arm 1: <i>Ultomiris</i> Q8W • Arm 2: Placebo 	<ul style="list-style-type: none"> • Primary endpoint: Complete TMA response • Secondary endpoints: Time to complete TMA response, Hematologic response, Renal response 	<ul style="list-style-type: none"> • FPCD: Q3 2021 • Data anticipated: 2023+
Phase III ALXN-1210-TM-313 NCT04543591	Thrombotic microangiopathy associated with haematopoietic stem cell transplant	184	<ul style="list-style-type: none"> • Arm 1: <i>Ultomiris</i> Q8W • Arm 2: Placebo 	<ul style="list-style-type: none"> • Primary endpoint: TMA response • Secondary endpoints: Time to TMA response, TMA relapse 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • Data anticipated: 2023+
Phase III ALXN-1210-TM-314 NCT04557735	Paediatric thrombotic microangiopathy associated with haematopoietic stem cell transplant	40	<ul style="list-style-type: none"> • Arm 1: <i>Ultomiris</i> administered once every 4-8 weeks 	<ul style="list-style-type: none"> • Primary endpoint: Proportion of participants with TMA response • Secondary endpoints: Time to TMA response, Proportion of participants with TMA relapse 	<ul style="list-style-type: none"> • FPCD: Q4 2020 • Data anticipated: 2023+
Phase II ALXN-1210-NEPH-202 NCT04564339	Proliferative lupus nephritis or immunoglobulin A nephropathy	120	<ul style="list-style-type: none"> • Arm 1: LN Cohort, <i>Ultomiris</i> • Arm 2: LN Cohort, Placebo • Arm 3: IgAN Cohort, <i>Ultomiris</i> • Arm 4: IgAN Cohort, Placebo 	<ul style="list-style-type: none"> • Primary endpoint: Both cohorts, percentage change in proteinuria from baseline to Week 26 • Secondary endpoints: Both cohorts, percentage change in proteinuria from baseline to Week 50 	<ul style="list-style-type: none"> • FPCD: Q1 2021 • Data anticipated: 2023+



Ultomiris (anti-complement C5 mAb)

Neurology

Approved medicines
Late-stage development
Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II/III ALXN-1210-ALS-308 NCT04248465	Amyotrophic lateral sclerosis	382	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in ALSFRS-R total score 	<ul style="list-style-type: none"> Trial halted post futility analysis Q3 2021
Phase III ALXN-1210-NMO-307 NCT04201262	Neuromyelitis optica spectrum disorder	58	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Q8W 	<ul style="list-style-type: none"> Primary endpoint: time to first adjudicated on-trial relapse 	<ul style="list-style-type: none"> FPCD: Q4 2019 LPCD: Q1 2021 Data anticipated: H1 2022
Phase III ALXN-1210-MG-306 NCT03920293	Generalised myasthenia gravis	175	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in MG-ADL total score at Week 26 	<ul style="list-style-type: none"> Data readout: Q2 2021 Primary endpoint met
Phase II/III ALXN1210-DM-310 NCT04999020	Dermatomyositis	180	<ul style="list-style-type: none"> Arm 1: <i>Ultomiris</i> Arm 2: placebo 	<ul style="list-style-type: none"> Primary endpoint : improvement response on International Myositis Assessment And Clinical Studies-Total Improvement Score (IMACS-TIS) 	<ul style="list-style-type: none"> FPCD: Q4 2021 Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



ALXN1840 (bis-choline tetrathiomolybdate)

Wilson disease

Trial	Population	Patients	Design	Endpoints	Status
Phase III WTX101-301 FoCus NCT03403205	Wilson Disease	215	<ul style="list-style-type: none"> Arm 1: ALXN1840 was administered orally for 48 weeks at doses ranging from 15 milligrams (mg) every other day (QOD) up to a titrated dose of 60 mg daily. Arm 2: Standard of care 	<ul style="list-style-type: none"> Primary endpoint: daily mean AUEC of dNCC Secondary endpoint: change from baseline in the UWDRS Part II total score 	<ul style="list-style-type: none"> Data readout: Q3 2021 Primary endpoint met
Phase II ALXN1840-WD-204 NCT04573309	Wilson Disease	10	<ul style="list-style-type: none"> Arm 1: Participants will be administered ALXN1840 at a dose of 15 milligrams (mg)/day on Day 1 through Day 28 and then increased to 30 mg/day on Day 29 through Day 39 	<ul style="list-style-type: none"> Primary endpoint: mean daily copper balance 	<ul style="list-style-type: none"> FPCD: Q3 2020 Data anticipated: H2 2022
Phase II ALXN1840-WD-205 NCT04422431	Wilson Disease	31	<ul style="list-style-type: none"> Arm 1: ALXN1840, Participants will be initiated at 15 milligrams once daily, then the dose will be increased to 30 milligrams once daily at Week 6. 	<ul style="list-style-type: none"> Primary endpoint: change from baseline at Week 48 in liver copper concentration 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: H2 2022



Andexxa (anti-factor Xa reversal)

Haematology

Trial	Population	Patients	Design	Endpoints	Status
Post Launch Phase IV I8-513 NCT03661528	Acute Intracranial Haemorrhage	1200	<ul style="list-style-type: none"> Arm 1: <i>Andexxa</i> Arm 2: Usual care Global trial	<ul style="list-style-type: none"> Primary endpoint: proportion of patients with good or excellent hemostatic efficacy as rated by an independent adjudication committee Secondary endpoint: change from baseline in anti-fXa activity 	<ul style="list-style-type: none"> FPCD: Q2 2019 Data anticipated: 2023+
Phase II 19-515 NCT04233073	Urgent surgery	100	<ul style="list-style-type: none"> Arm 1: <i>Andexxa</i> 	<ul style="list-style-type: none"> Primary endpoint: proportion of patients with good or excellent intraoperative hemostatic efficacy as determined by the surgeon's assessment and confirmed by an independent adjudication committee Secondary endpoint: percent change from baseline in anti-factor Xa activity 	<ul style="list-style-type: none"> FPCD: Q2 2021 Data anticipated: 2023



CAEL-101 (fibril-reactive mAb)

AL amyloidosis

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III CAEL101-302 NCT04512235	Mayo stage IIIa amyloidosis	267	<ul style="list-style-type: none"> Arm 1: CAEL-101 combined with SoC for plasma cell dyscrasia (PCD) Arm 2: placebo combined with SoC for PCD 	<ul style="list-style-type: none"> Primary: time from first dose of trial drug until death or end of trial Secondary: change in distance walked during a six-minute walk test, and quality of life measures 	<ul style="list-style-type: none"> FPCD: Q4 2020 Data anticipated: 2023+
Phase III CAEL101-301 NCT04504825	Mayo stage IIIb amyloidosis	110	<ul style="list-style-type: none"> Arm 1: CAEL-101 combined with SoC for PCD Arm 2: placebo combined with SoC for PCD 	<ul style="list-style-type: none"> Primary: time from first dose of trial drug until death or end of trial Secondary: change in distance walked during a six-minute walk test, and quality of life measures 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023+
Phase II CAEL101-203 NCT04304144	Mayo Stage I, Stage II and Stage IIIa amyloidosis	25	<ul style="list-style-type: none"> Arm 1: CAEL-101 combined with SoC CyBorD Arm 2: placebo combined with SoC CyBorD and daratumumab 	<ul style="list-style-type: none"> Primary: dose toxicity – occurrence of dose limiting toxicity (DLT) during the first 4 weeks of therapy Secondary: area under the plasma curve concentration versus time curve (AUC) 	<ul style="list-style-type: none"> FPCD: Q1 2020 Data anticipated: 2023

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



acoramidis (ALXN2060)

ATTR-CM

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN2060-TAC-302 NCT04622046	ATTR-CM	22	<ul style="list-style-type: none">Arm 1: 800 mg acoramidis (ALXN2060) administered twice daily	<ul style="list-style-type: none">Primary endpoint: Change from Baseline to Month 12 of Treatment In Distance Walked During the Six-minute Walk Test, All-cause mortality and cardiovascular-related hospitalization over a 30-month period	<ul style="list-style-type: none">FPCD: Q4 2020Data anticipated: H2 2022

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



danicopan (ALXN2040 Factor D inhibitor)

Haematology & ophthalmology

Trial	Population	Patients	Design	Endpoints	Status
Phase III ALXN2040-PNH-301 NCT04469465	PNH with clinically relevant extravascular haemolysis	84	<ul style="list-style-type: none"> Arm 1: danicopan (ALXN2040) + C5 Inhibitor Arm 2: placebo + C5 Inhibitor 	<ul style="list-style-type: none"> Primary endpoint: change from baseline in haemoglobin at week 12 Secondary endpoint: percentage of participants with transfusion avoidance 	<ul style="list-style-type: none"> FPCD: Q1 2021 Data anticipated: 2023
Phase II ALXN2040-GA-201 NCT05019521	Geographic atrophy	330	<ul style="list-style-type: none"> Arms 1-3: danicopan (ALXN2040) dosed at 100-400mg daily Arm 4: placebo 	<ul style="list-style-type: none"> Primary endpoint: mean rate of change from baseline at week 52 in the square root of total GA lesion area in the trial eye as measured by FAF 	<ul style="list-style-type: none"> FPCD: Q3 2021 Data anticipated: 2023+



**Rare Disease –
early-stage development**



ALXN1720 (anti-C5 bi-specific minibody)

Neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1720-HV-101 NCT04920370	Healthy Volunteers	96	<ul style="list-style-type: none">• Single & Multiple Ascending Dosing of ALXN1720 via s.c. and i.v.• Placebo	<ul style="list-style-type: none">• Primary: Incidence of TEAEs and SAEs	<ul style="list-style-type: none">• FPCD: Q3 2019• Data anticipated: H1 2022

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



ALXN2050 (factor D inhibitor)

Haematology, nephrology, neurology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase II ACH228-110 NCT04170023	PNH	26	<ul style="list-style-type: none">Arm 1: ALXN2050 Monotherapy, with groups including treatment-naïve, C5 inhibitor treatment experienced, and patients previously receiving danicopan (ALXN2040)	<ul style="list-style-type: none">Primary endpoint: Change in haemoglobin relative to baselineSecondary endpoints: Number of participants who have transfusion avoidance, Change in lactate dehydrogenase relative to baseline	<ul style="list-style-type: none">FPCD: Q4 2019Data anticipated: 2023+

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



ALXN1820 (anti-properdin)

Haematology

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase I ALXN1820-HV-101 NCT04631562	Healthy Volunteers	80	<ul style="list-style-type: none">• Arm 1: ALXN1820 administered s.c. or i.v., multiple ascending doses• Arm 2: Placebo	<ul style="list-style-type: none">• Primary: Participants with TEAEs	<ul style="list-style-type: none">• FPCD: Q1 2021• Data anticipated: 2023

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



ALXN1850 (next generation asfotase alfa)

Hypophosphatasia

Approved medicines

Late-stage development

Early development

Trial	Population	Patients	Design	Endpoints	Status
Phase 1 ALXN1850-HPP-101 NCT04980248	HPP	15	<ul style="list-style-type: none">Arm 1: ALXN1850, 3 cohorts at low, medium, and high dosages	<ul style="list-style-type: none">Primary: Incidence of TEAEs and TESAEs	<ul style="list-style-type: none">FPCD: Q3 2021Data anticipated: H2 2022

Oncology

CVRM

R&I

Other

COVID-19

Rare Disease



List of abbreviations

14C	Radioactive isotope of carbon, Carbon 14
1L, 2L, 3L	1st, 2nd or 3rd line
5-FU	5-fluorouracil
A2AR	Adenosine A2A receptor
ACQ	Asthma control questionnaire
ACR	American college of rheumatology response scoring system
ADA	Anti-drug antibodies
ADC	Antibody-drug conjugate
ADP	Adenosine diphosphate
AE	Adverse event
aHUS	Atypical haemolytic uraemic syndrome
AI	Auto-injector
AKT	Protein kinase B
ALK	Anaplastic large-cell lymphoma kinase
ALSFRS-R	Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised
APFS	Accessorised pre-filled syringe
AQLQ	Asthma quality of life questionnaire
AS	Albuterol sulphate
ATM	Ataxia-telangiectasia mutated kinase
ATR	Ataxia telangiectasia and rad3-related protein
ATTR-CM	Transthyretin amyloid cardiomyopathy
AUC	Area under curve
AUEC	Area under the effect-time curve
B7RP	B7-related protein-1
BA	Bioavailability
BAFF	B-cell activating factor
BCG	Bacillus Calmette–Guérin
BCMA	B-cell maturation antigen
BDA	Budesonide albuterol
BFF	Budesonide and formoterol fumarate
BGF	Budesonide, glycopyrronium and formoterol fumarate
BICR	Blinded independent central review
BID	Bis in die (twice per day)
BIG	Big ten cancer research consortium
BMD	Bone mineral density
BMFI	Bone metastasis-free interval
BMI	Body mass index
BRCAwt	Breast cancer wild-type gene
BRD4	Bromodomain-containing protein 4
BTC	Biliary tract carcinoma

BTK	Bruton's tyrosine kinase
CA-125	Cancer antigen 125
CAD	Coronary artery disease
CBR	Clinical benefit rate
CD	Cluster of differentiation
CDK	Cyclin-dependent kinase
CE	Clinically evaluable
CHD	Coronary heart disease
Chemo	Chemotherapy
CHF	Chronic heart failure
CKD	Chronic kidney disease
CLL	Chronic lymphocytic leukaemia
CMAx	Maximum observed plasma concentration
C-MET	Tyrosine-protein kinase Met
CNS	Central nervous system
COPD	Chronic obstructive pulmonary disease
CR	Complete response
CRC	Colorectal cancer
CrCl	Creatinine clearance
CRR	Complete response rate
CTC	Circulating tumour cell
CTLA-4	Cytotoxic T-lymphocyte-associated antigen 4
CV	Cardiovascular
CVOT	Cardiovascular outcomes trial
CVRM	Cardiovascular renal and metabolism
CXCR2	C-X-C Motif chemokine receptor 2
DB	Double blind
DC	Disease control
DCR	Disease control rate
DDI	Drug-drug Interaction
dECG	Differentiated electrocardiogram
DFS	Disease free survival
DLBCL	Diffuse large B-cell lymphoma
DLT	Dose-limiting toxicity
DMARDs	Disease-modifying antirheumatic drugs
DNA	Deoxyribonucleic acid
DoCR	Durability of complete response
dNCC	Directly Measured Non-ceruloplasmin-bound Copper
DoR	Duration of response
DPI	Dry powder inhaler

DRFI	Disease recurrence-free interval
DXA	Dual energy X-ray absorptiometry
EBRT	External beam radiation therapy
ECG	Electrocardiogram
EFS	Event-free survival
eGFR	Estimated glomerular filtration rate
EGFR	Epidermal growth factor receptor
ER	Oestrogen receptor
ERK	Extracellular signal-regulated kinase
ESCC	Esophageal squamous cell carcinoma
ESR	Externally sponsored trial
ESR1	Oestrogen receptor 1
ET	Endocrine therapy
FAF	Fundus Autofluorescence
FDC	Fixed-dose combination
FeNO	Fractional nitric oxide concentration in exhaled breath
FEV	Forced-expiratory volume
FGFR	Fibroblast growth factor receptor
FLAP	5-lipoxygenase-activating protein
FPDC	First patient commenced dosing
FPG	Fasting plasma glucose
GA	Gestational age
GA	Geographic atrophy
GBM	Glioblastoma
gBRCAm or tBRCAm	Germline or tumour (somatic) BRCA mutation
GEJ	Gastric/gastro-oesophageal junction
GFF	Glycopyrronium and formoterol fumarate
GLP-1	Glucagon-like peptide-1
GMFRs	Geometric mean fold rises
GMTs	Geometric mean titers
hADME	Human mass balance
HAI	Haemagglutination-inhibition
HbA1c	Haemoglobin A1c
HCC	Hepatocellular carcinoma
HD	High dose
HDL-C	High-density lipoprotein cholesterol
HER2	Human epidermal growth factor receptor 2
HF	Heart failure
HFpEF	Heart failure with preserved ejection fraction



List of abbreviations

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



List of abbreviations

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





Clinical trials appendix

Q1 2021 results update

