PIPELINE

Novartis is consistently rated as having one of the industry's most respected development pipelines, with more than 200 projects in clinical development, as of December 31, 2015.

Many of these projects, which include new molecular entities as well as additional indications and different formulations for marketed products, are for potentially best-in-class or first-in-class medicines that could significantly advance treatment standards for patients worldwide. This table provides an overview of selected projects in confirmatory development.

We use the traditional pipeline model as a platform (e.g., Phase I-III). However, we have tailored the process to be simpler, more flexible and more efficient.

GLOSSARY

Project/product Project refers to the Novartis reference code (combination of three letters and three numbers) used for projects in development. Product refers to the brand name for a marketed product.

Common name Official international non-proprietary name or generic name for an individual molecular entity as designated by the World Health Organization

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MAJOR DEVELOPMENT PROJECTS

Project/product	Division	Common name	Mechanism of action	Potential indication/disease area	Route of administration	Planned filing dates[1,2]	PHASE I	PHASE II	PHASE III	SUBMISSION
ONCOLOGY										
ABL001	Pharmaceuticals	–	BCR-ABL inhibitor	Chronic myeloid leukemia	Oral	≥2020	PHASE I			
ASB183	Pharmaceuticals	afuresertib	AKT inhibitor	Solid and hematologic tumors	Oral	≥2020	PHASE I			
LJM716	Pharmaceuticals	elgemtumab	HER3 mAb[3]	Solid tumors	Intravenous infusion	≥2020	PHASE I			
PIM447	Pharmaceuticals	–	Pan-PIM inhibitor	Hematologic tumors	Oral	≥2020	PHASE I			
EGF816	Pharmaceuticals	–	Epidermal growth factor receptor inhibitor	Solid tumors	Oral	2018		PHASE II		
BGJ398	Pharmaceuticals	infigratinib	Pan-FGF receptor kinase inhibitor	Solid tumors	Oral	≥2020		PHASE II		
Tafinlar + Mekinist	Pharmaceuticals	dabrafenib + trametinib	BRAF inhibitor + MEK[4] inhibitor	BRAF V600+ NSCLC,[2] BRAF V600+ melanoma (adjuvant), BRAF V600+ colorectal cancer	Oral	2016		PHASE II		
INC280	Pharmaceuticals	capmatinib	c-MET inhibitor	Non-small cell lung cancer	Oral	2018		PHASE II		
BKM120	Pharmaceuticals	buparlisib	PI3K[5] inhibitor	Metastatic breast cancer, hormone receptor-positive, aromatase inhibitor resistant/mTOR naïve, 2nd line (+ fulvestrant) [lead indication]; metastatic breast cancer, hormone receptor-positive, aromatase inhibitor and mTOR inhibitor resistant, 3rd line (+ fulvestrant); solid tumors	Oral	2016			PHASE III	
BYL719	Pharmaceuticals	alpelisib	PI3Kα[6] inhibitor	Hormone receptor-positive, HER2-negative advanced breast cancer (postmenopausal women), 2nd line (+ fulvestrant) [lead indication]; solid tumors	Oral	2019			PHASE III	
Tasigna	Pharmaceuticals	nilotinib	BCR-ABL inhibitor	Chronic myeloid leukemia treatment-free remission	Oral	2016			PHASE III	
LCI699	Pharmaceuticals	osilodrostat	Aldosterone synthase inhibitor	Cushing's disease	Oral	2017			PHASE III	
LEE011	Pharmaceuticals	ribociclib	CDK4/6[7] inhibitor	Hormone receptor-positive, HER2-negative advanced breast cancer (postmenopausal women), 1st line (+ letrozole) [lead indication]; hormone receptor-positive, HER2-negative advanced breast cancer (premenopausal women), 1st line (+ tamoxifen + goserelin or NSAI[8] + goserelin); hormone receptor-positive, HER2-negative advanced breast cancer (postmenopausal women), 1st/2nd line (+ fulvestrant); solid tumors	Oral	2016			PHASE III	
PKC412	Pharmaceuticals	midostaurin	Signal transduction inhibitor	Acute myeloid leukemia [lead indication], aggressive systemic mastocytosis	Oral	2016			PHASE III	
Signifor LAR (SOM230)	Pharmaceuticals	pasireotide	Somatostatin analogue	Cushing's disease	Long-acting release, intramuscular injection	2016			PHASE III	
Zykadia (LDK378)	Pharmaceuticals	ceritinib	ALK[9] inhibitor	ALK[9]+ advanced non-small cell lung cancer (1st line, treatment naïve),[2] ALK[9]+ advanced non-small cell lung cancer (brain metastases)	Oral	2017			PHASE III	
Votrient	Pharmaceuticals	pazopanib	Angiogenesis inhibitor	Renal cell carcinoma (adjuvant)	Oral	2016			PHASE III	
Arzerra	Pharmaceuticals	ofatumumab	Anti-CD20 mAb[3]	Chronic lymphocytic leukemia (extended treatment),[2] chronic lymphocytic leukemia (relapse), non-Hodgkin's lymphoma (refractory)	Intravenous infusion	US registration EU registration				SUBMISSION
Afinitor/Votubia (RAD001)	Pharmaceuticals	everolimus	mTOR[10] inhibitor	Non-functioning GI and lung neuroendocrine tumors,[2] tuberous sclerosis complex seizures, DLBCL[11]	Oral	US registration EU registration				SUBMISSION
Promacta/Revolade	Pharmaceuticals	eltrombopag	Thrombopoietin receptor agonist	Pediatric immune thrombocytopenia	Oral/oral suspension	US approved EU registration				SUBMISSION
Jadenu *Exjade* film-coated tablet (FCT)	Pharmaceuticals	deferasirox	Iron chelator	Iron overload	Oral FCT	US approved EU registration				SUBMISSION
CARDIOVASCULAR AND METABOLISM										
ACZ885	Pharmaceuticals	canakinumab	Anti-interleukin-1ß monoclonal antibody	Secondary prevention of cardiovascular events	Subcutaneous injection	2017			PHASE III	
RLX030	Pharmaceuticals	serelaxin	Recombinant form of human relaxin-2 hormone	Acute heart failure	Intravenous infusion	2017			PHASE III	
Entresto (LCZ696)	Pharmaceuticals	valsartan, sacubitril (as sodium salt complex)	Angiotensin receptor, neprilysin inhibitor	Chronic heart failure with preserved ejection fraction,[2] post-acute myocardial infarction	Oral	2019			PHASE III	

[1] Filings that have received approval in either the US or EU but are awaiting approval in the other market
[2] Phase and planned filing dates refer to lead indication in development.
[3] Monoclonal antibody
[4] Combination of mitogen-activated protein kinase and extracellular signal-regulated kinase
[5] Phosphoinositide 3-kinase inhibitor
[6] Phosphoinositide 3-kinase alpha inhibitor
[7] Cyclin-dependent kinase 4/6
[8] Non-steroidal aromatase inhibitor
[9] Anaplastic lymphoma kinase
[10] Mammalian target of rapamycin
[11] Diffuse large B-cell lymphoma

PIPELINE
continued

Mechanism of action Specific biochemical interaction with a molecular target such as a receptor or enzyme, through which a drug substance produces its pharmacological effect

Potential indication/indications Disease or condition for which a compound or marketed product is in development and is being studied as a potential therapy

Route of administration Path by which a medicinal preparation is administered into the body, such as oral, subcutaneous or intravenous

Phase I First clinical trials of a new compound, generally performed in a small number of healthy human volunteers, to assess the clinical safety and tolerability, as well as metabolic and pharmacologic properties of the compound

Phase II Clinical studies with patients who have the target disease, with the aim of continuing the Phase I safety assessment in a larger group, assessing the efficacy of the drug in the patient population, and determining the appropriate doses for further evaluation

Phase III Large-scale clinical studies with several hundred to several thousand patients, which are conducted to establish the safety and efficacy of the drug-specific indications for regulatory approval. Phase III trials also may be used to compare a new drug against a current standard of care to evaluate the overall benefit-risk relationship of the new medicine.

Glossary continued on page 56

MAJOR DEVELOPMENT PROJECTS

Project/product	Division	Common name	Mechanism of action	Potential indication/disease area	Route of administration	Planned filing dates[1,2]	PHASE I	PHASE II	PHASE III	SUBMISSION
RESPIRATORY										
QAX576	Pharmaceuticals	–	Anti-interleukin-13 monoclonal antibody	Allergic diseases	Subcutaneous injection	≥2020		PHASE II		
QMF149	Pharmaceuticals	indacaterol, mometasone furoate (in fixed-dose combination)	Long-acting beta2-agonist and inhaled corticosteroid	Asthma	Inhalation	2018			PHASE III	
QAW039	Pharmaceuticals	fevipiprant	CRTH2 antagonist	Asthma	Oral	2019			PHASE III	
QVM149	Pharmaceuticals	indacaterol, mometasone furoate, glycopyrronium bromide (in fixed-dose combination)	Long-acting beta2-agonist, long-acting muscarinic antagonist and inhaled corticosteroid	Asthma	Inhalation	2018			PHASE III	
IMMUNOLOGY AND DERMATOLOGY										
CJM112	Pharmaceuticals	–	Anti-interleukin-17 monoclonal antibody	Immune disorders	Subcutaneous injection	≥2020		PHASE II		
QAW039	Pharmaceuticals	fevipiprant	CRTH2 antagonist	Atopic dermatitis	Oral	≥2020		PHASE II		
LJN452	Pharmaceuticals	–	FXR agonist	Non-alcoholic steatohepatitis	Oral	≥2020		PHASE II		
VAY736	Pharmaceuticals	–	Anti-BAFF (B-cell-activating factor) antibody	Primary Sjoegren's syndrome	Subcutaneous injection	≥2020		PHASE II		
QGE031	Pharmaceuticals	ligelizumab	High-affinity anti-IgE monoclonal antibody	Chronic spontaneous urticaria/ inducible urticaria	Subcutaneous injection	≥2020		PHASE II		
Ilaris (ACZ885)	Pharmaceuticals	canakinumab	Anti-interleukin-1ß monoclonal antibody	Hereditary periodic fevers	Subcutaneous injection	2016			PHASE III	
Cosentyx (AIN457)	Pharmaceuticals	secukinumab	Anti-interleukin-17 monoclonal antibody	Ankylosing spondylitis,[2] psoriatic arthritis,[2] non-radiographic axial spondyloarthritis	Subcutaneous injection	US registration EU approved				SUBMISSION
NEUROSCIENCE										
CAD106	Pharmaceuticals	–	Beta-amyloid-protein therapy	Alzheimer's disease	Intramuscular injection	≥2020		PHASE II		
CNP520	Pharmaceuticals	–	BACE inhibitor	Alzheimer's disease	Oral	≥2020		PHASE II		
EMA401	Pharmaceuticals	–	Angiotensin II receptor antagonist	Neuropathic pain	Oral	≥2020		PHASE II		
OMB157	Pharmaceuticals	ofatumumab	Anti-CD-20 monoclonal antibody	Relapsing multiple sclerosis	Subcutaneous injection	2019		PHASE II		
BAF312	Pharmaceuticals	siponimod	Sphingosine-1-phosphate receptor modulator	Secondary progressive multiple sclerosis	Oral	2019			PHASE III	
Gilenya	Pharmaceuticals	fingolimod	Sphingosine-1-phosphate receptor modulator	Chronic inflammatory demyelinating polyradiculoneuropathy	Oral	2017			PHASE III	
AMG 334	Pharmaceuticals	–	Selective CGRP receptor antagonist	Migraine	Subcutaneous injection				PHASE III	
BYM338	Pharmaceuticals	bimagrumab	Inhibitor of activin type II receptor	Sporadic inclusion body myositis [lead indication], hip fracture, sarcopenia	Intravenous infusion	2016			PHASE III	
CELL AND GENE THERAPY										
CTL019	Pharmaceuticals	tisagenlecleucel-T	CD19-targeted chimeric antigen receptor T-cell immunotherapy	Pediatric acute lymphoblastic leukemia [lead indication], diffuse large B-cell lymphoma	Intravenous infusion	2016		PHASE II		
FCR001	Pharmaceuticals	–	Inducing stable donor chimerism and immunological tolerance	Renal transplant	Intravenous infusion	≥2020		PHASE II		
HSC835	Pharmaceuticals	–	Stem cell regeneration	Stem cell transplantation	Intravenous infusion	≥2020		PHASE II		
INFECTIOUS DISEASES										
KAF156	Pharmaceuticals	–	Imidazolopiperazines derivative	Malaria	Oral	2019		PHASE II		
KAE609	Pharmaceuticals	cipargamin	PfATP4 inhibitor	Malaria	Oral	≥2020		PHASE II		
EXE844b	Alcon	finafloxacin	Anti-infective	Otitis media-tympanostomy tube surgery	Topical	2016 US			PHASE III	

[1] Filings that have received approval in either the US or EU but are awaiting approval in the other market
[2] Phase and planned filing dates refer to lead indication in development.

PIPELINE
continued

Advanced development Medical device project for which a positive proof of concept has been established and studies are being conducted to establish the safety, efficacy or performance to address regulatory requirements for obtaining marketing authorization

Submission An application for marketing approval has already been submitted to one or both of the following regulatory agencies: the US Food and Drug Administration (FDA), the European Medicines Agency (EMA). Novartis has not yet received marketing authorization from both regulatory agencies. The application contains comprehensive data and information gathered during human clinical trials and animal studies conducted through the various phases of drug development.

MAJOR DEVELOPMENT PROJECTS

Project/product	Division	Common name	Mechanism of action	Potential indication/disease area	Route of administration	Planned filing dates[1,2]	PHASE I	PHASE II	PHASE III	SUBMISSION
OPHTHALMOLOGY										
Lucentis	Pharmaceuticals	ranibizumab	Anti-vascular endothelial growth factor (VEGF) monoclonal antibody fragment	Choroidal neovascularization,[12] retinopathy of prematurity	Intravitreal injection	2016			PHASE III	
OAP030 (Fovista®)	Pharmaceuticals	pegpleranib	Aptamer anti-platelet-derived growth factor	Neovascular age-related macular degeneration	Intravitreal injection	2017			PHASE III	
Jetrea ready-diluted injection	Alcon	ocriplasmin	Alpha-2 antiplasmin reducer	Vitreomacular traction	Intravitreal injection	2017 Japan			PHASE III	
RTH258	Alcon	brolucizumab	Anti-VEGF single-chain antibody fragment	Wet age-related macular degeneration	Intravitreal injection	≥2018			PHASE III	
Ilevro ophthalmic suspension	Alcon	nepafenac (0.3%)	Anti-inflammatory	Postsurgical macular edema in patients with diabetes	Topical	Submitted EU 2018 US			PHASE III	
AcrySof IQ ReSTOR Toric 2.5 D IOL	Alcon	–	Multifocal, aspheric and cylinder-correcting intraocular lens	Cataractous lens replacement with or without presbyopia, and with astigmatism	Surgical	2016 US	ADVANCED DEVELOPMENT			
AOSept Plus/ *Clear Care* Plus with *HydraGlyde*	Alcon	–	Disinfection and cleaning	Contact lens care	Lens care	2017 Japan	ADVANCED DEVELOPMENT			
AcrySof IQ Aspheric IOL with *UltraSert*	Alcon	–	Pre-loaded intraocular lens delivery device	Cataractous lens replacement	Surgical	Submitted Japan	ADVANCED DEVELOPMENT			SUBMISSION
AcrySof IQ ReSTOR Toric 3.0 D IOL	Alcon	–	Multifocal, aspheric and cylinder-correcting intraocular lens	Cataractous lens replacement with or without presbyopia, and with astigmatism	Surgical	Submitted US	ADVANCED DEVELOPMENT			SUBMISSION
BIOSIMILARS										
GP2013	Sandoz	rituximab	Anti-CD20 antibody	Non-Hodgkin's lymphoma, chronic lymphocytic leukemia, rheumatoid arthritis, granulomatosis with polyangiitis (also known as Wegener's granulomatosis), and microscopic polyangiitis and others (same as originator)	Intravenous				PHASE III	
GP2017	Sandoz	adalimumab	TNF-α inhibitor	Arthritides (rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis), plaque psoriasis and others (same as originator)	Subcutaneous				PHASE III	
HX575	Sandoz	epoetin alfa	Erythropoiesis-stimulating agent	Anemia in chronic kidney disease, chemotherapy-induced anemia and others (same as originator)	Subcutaneous and intravenous	US			PHASE III	
HX575 s.c.	Sandoz	epoetin alfa	Erythropoiesis-stimulating agent	Anemia in chronic kidney disease	Subcutaneous	Submitted EU (extension nephrology, appproved as *Binocrit* since 2007)				SUBMISSION
GP2015	Sandoz	etanercept	TNF-α inhibitor	Arthritides (rheumatoid arthritis, ankylosing spondylitis, psoriatic arthritis), plaque psoriasis and others (same as originator)	Subcutaneous	Submitted US Submitted EU				SUBMISSION
LA-EP2006	Sandoz	pegfilgrastim	Pegylated granulocyte colony-stimulating factor	Chemotherapy-induced neutropenia and others (same as originator)	Subcutaneous	Submitted US				SUBMISSION

[1] Filings that have received approval in either the US or EU but are awaiting approval in the other market
[2] Phase and planned filing dates refer to lead indication in development.
[12] Choroidal neovascularization secondary to conditions other than age-related macular degeneration and pathologic myopia