Pfizer Pipeline

As of January 28, 2020

Disclaimer

- As some programs are still confidential, some candidates may not be identified in this list. In these materials, Pfizer discloses Mechanism of Action (MOA) information for some candidates in Phase 1 and for all candidates from Phase 2 through regulatory approval. With a view to expanding the transparency of our pipeline, Pfizer is including new indications or enhancements, which target unmet medical need or represent significant commercial opportunities. The information contained on these pages is correct as of January 28, 2020.

- Visit Pfizer.com/pipeline, Pfizer's online database where you can learn more about our portfolio of new medicines and find out more about our Research and Development efforts around the world.

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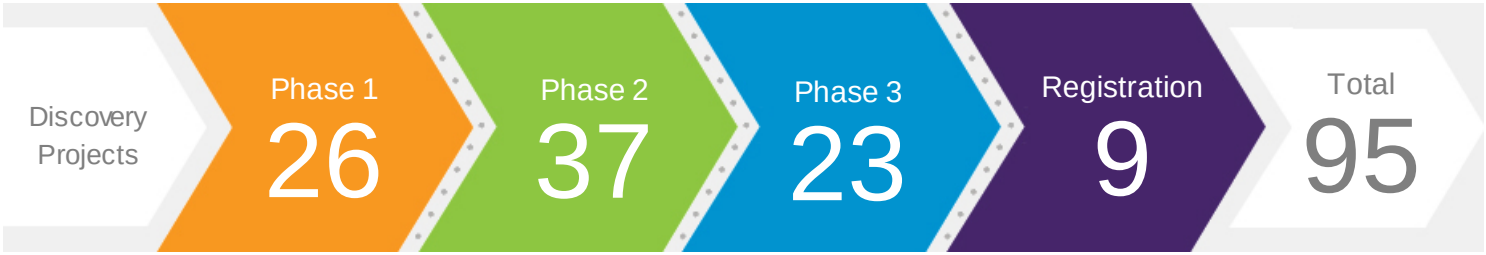
Pfizer Pipeline Snapshot



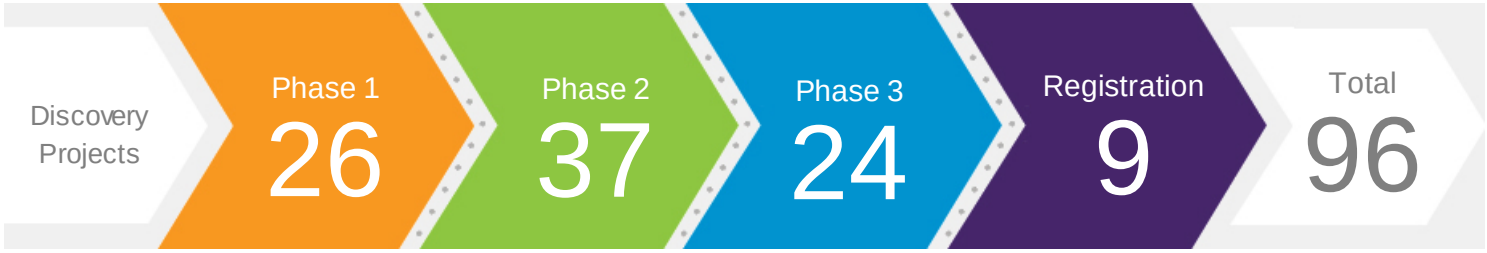
Pfizer Pipeline Snapshot as of January 28, 2020

Pipeline represents progress of R&D programs as of January 28, 2020

- 9 programs advanced or are new
- 7 programs discontinued since last update
- Included are 58 NMEs, 34 additional indications, plus 3 biosimilars

Recent Approval

- XTANDI® (enzalutamide) for the treatment of patients with metastatic castration-sensitive prostate cancer (U.S.)
- ABRILADA™ (adalimumab-afzb), as a biosimilar to Humira®(1) (adalimumab), for the treatment of certain patients with rheumatoid arthritis, juvenile idiopathic arthritis, psoriatic arthritis, ankylosing spondylitis, adult Crohn's disease, ulcerative colitis and plaque psoriasis (U.S.)
- XELJANZ® (tofacitinib) prolonged release in combination with methotrexate for the treatment of moderate to severe active rheumatoid arthritis in adult patients who have responded inadequately to, or who are intolerant to one or more disease-modifying antirheumatic drugs (E.U.)



Pfizer Pipeline Snapshot as of October 29, 2019

Pipeline represents progress of R&D programs as of October 29, 2019

- 11 programs advanced or are new
- 7 programs discontinued since last update
- Included are 55 NMEs, 38 additional indications, plus 3 biosimilars

Recent Approval

- BAVENCIO® (avelumab) in combination with INLYTA (axitinib) for the first-line treatment of adult patients with advanced renal cell carcinoma (E.U.)

(1) Humira® is a registered U.S. trademark of Abbvie Biotechnology Ltd.

Inflammation and Immunology (1 of 2)

Discovery Projects	Phase 1	Phase 2	Phase 3	Registration	Total
	3	16	3	2	24

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
crisaborole (PF-06940799)	PDE4 Inhibitor	Atopic Dermatitis (E.U.)	Registration	New Molecular Entity
PF-06410293, a potential biosimilar to Humira® (adalimumab)	Tumor Necrosis Factor Inhibitor	Rheumatoid Arthritis (Biosimilar) (E.U.)	Registration	Biosimilar
abrocitinib (PF-04965842)	JAK Inhibitor	Atopic Dermatitis (BREAKTHROUGH)	Phase 3	New Molecular Entity
PF-06651600	JAK3/TEC	Alopecia Areata (BREAKTHROUGH)	Phase 3	New Molecular Entity
Xeljanz (tofacitinib)	JAK Inhibitor	Ankylosing Spondylitis	Phase 3	Product Enhancement
Dekavil	IL-10	Rheumatoid Arthritis (Biologic)	Phase 2	New Molecular Entity
Dekavil	IL-10	Ulcerative Colitis (Biologic)	Phase 2	Product Enhancement
PF-06480605	TNFSF15 Blocker	Ulcerative Colitis (Biologic)	Phase 2	New Molecular Entity
PF-06650833	IRAK4	Rheumatoid Arthritis	Phase 2	New Molecular Entity
PF-06651600	JAK3/TEC	Rheumatoid Arthritis	Phase 2	Product Enhancement
▶ PF-06650833 PF-06700841 PF-06826647	IRAK4 TYK2/JAK1 TYK2 Inhibitor	Hidradenitis Suppurativa	Phase 2	Product Enhancement
PF-06651600 PF-06700841	JAK3/TEC TYK2/JAK1	Ulcerative Colitis	Phase 2	New Molecular Entity
PF-06651600 PF-06700841	JAK3/TEC TYK2/JAK1	Crohn's Disease	Phase 2	Product Enhancement
PF-06651600 PF-06700841	JAK3/TEC TYK2/JAK1	Vitiligo	Phase 2	Product Enhancement
PF-06700841	TYK2/JAK1	Psoriatic Arthritis	Phase 2	Product Enhancement

▶ Indicates that the project is either new or has progressed in phase since the previous portfolio update of Pfizer.com

- Regulatory Designations – See Definitions in Backup
- Humira® is a registered U.S. trademark of Abbvie Biotechnology Ltd.

Inflammation and Immunology (2 of 2)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
PF-06700841	TYK2/JAK1	Alopecia Areata	Phase 2	Product Enhancement
PF-06700841	TYK2/JAK1	Lupus	Phase 2	Product Enhancement
PF-06700841	Topical TYK2/JAK1	Atopic Dermatitis	Phase 2	New Molecular Entity
PF-06700841	Topical TYK2/JAK1	Psoriasis	Phase 2	New Molecular Entity
PF-06823859	interferon, beta 1, fibroblast (IFNB1) Blocker	Inflammatory Disorders (Biologic)	Phase 2	New Molecular Entity
PF-06826647	TYK2 Inhibitor	Psoriasis	Phase 2	New Molecular Entity
PF-06826647	TYK2 Inhibitor	Ulcerative Colitis	Phase 1	Product Enhancement
PF-06835375	Chemokine Inhibitor	Lupus (Biologic)	Phase 1	New Molecular Entity
▶ PF-07038124	Topical PDE4 Inhibitor	Atopic Dermatitis	Phase 1	New Molecular Entity

▶ Indicates that the project is either new or has progressed in phase since the previous portfolio update of Pfizer.com

Internal Medicine

Discovery Projects	Phase 1	Phase 2	Phase 3	Registration	Total
	5	4	1	0	10

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
tanezumab	Nerve Growth Factor Inhibitor	OA Signs and Symptoms (FAST TRACK), Chronic Low Back Pain (FAST TRACK), Cancer Pain (Biologic)	Phase 3	New Molecular Entity
PF-05221304	Acetyl CoA-Carboxylase (ACC) Inhibitor	Non-Alcoholic Steatohepatitis (NASH) with Liver Fibrosis (FAST TRACK)	Phase 2	New Molecular Entity
PF-06835919	Ketohexokinase (KHK) Inhibitor	Non-Alcoholic Steatohepatitis (NASH)	Phase 2	New Molecular Entity
PF-07055341	ACCi and DGAT2 Combination	Combo of PF-05221304 and PF-06865571 for Non-Alcoholic Steatohepatitis (NASH)	Phase 2	New Molecular Entity
▶ PF-07285557	Angiopoietin Like 3 (ANGPTL3)	Cardiovascular Risk Reduction, Severe Hypertriglyceridemia	Phase 2	New Molecular Entity
PF-06865571	Diacylglycerol O-Acyltransferase 2 (DGAT2) Inhibitor	Non-Alcoholic Steatohepatitis (NASH)	Phase 1	New Molecular Entity
PF-06882961	Glucagon-like peptide 1 receptor (GLP-1R) Agonist	Diabetes Mellitus-Type 2 and Obesity	Phase 1	New Molecular Entity
PF-06946860	Growth Factor Blocker	Cachexia (Biologic)	Phase 1	New Molecular Entity
▶ PF-06842874	CDK 4,6 Inhibitor	Pulmonary Arterial Hypertension	Phase 1	New Molecular Entity
▶ PF-07081532	Glucagon-like peptide 1 receptor (GLP-1R) Agonist	Diabetes Mellitus-Type 2 and Obesity	Phase 1	New Molecular Entity

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- Regulatory Designations – See Definitions in Backup

Oncology (1 of 3)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
PF-06881894, a potential biosimilar to Neulasta® (pegfilgrastim)	Human Granulocyte Colony Stimulating Factor	Neutropenia in patients undergoing cancer chemotherapy (Biosimilar) (U.S., E.U.)	Registration	Biosimilar
PF-05280586, a potential biosimilar to Rituxan® /MabThera® (rituximab)	CD20 antigen antagonist	Follicular Lymphoma (Biosimilar) (E.U.)	Registration	Biosimilar
Daurismo (glasdegib)	SMO (smoothened) antagonist	Combo w/low-dose cytarabine (LDAC) for Acute Myeloid Leukemia (E.U.)	Registration	New Molecular Entity
Xtandi (enzalutamide)	Androgen receptor inhibitor	Metastatic Hormone Sensitive Prostate Cancer (E.U.)	Registration	Product Enhancement
▶ Braftovi (encorafinib) + Erbitux® (cetuximab)	*BRAF* kinase inhibitor	2nd or 3rd Line BRAF-mutant Metastatic Colorectal Cancer (PRIORITY REVIEW – U.S., E.U.)	Registration	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	1st Line Non-Small Cell Lung Cancer (Biologic)	Phase 3	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	1st Line Urothelial Cancer (Biologic)	Phase 3	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Locally Advanced Squamous Cell Carcinoma of the Head and Neck (Biologic)	Phase 3	Product Enhancement
Daurismo (glasdegib)	SMO (smoothened) antagonist	Combo w/azacytidine in Acute Myeloid Leukemia (ORPHAN - U.S., E.U.)	Phase 3	Product Enhancement
Ibrance (palbociclib)	CDK 4,6 kinase inhibitor	High Risk Early Breast Cancer	Phase 3	Product Enhancement
Ibrance (palbociclib)	CDK 4,6 kinase inhibitor	Early Breast Cancer in Adjuvant Setting	Phase 3	Product Enhancement
▶ PF-06801591 + Bacillus Calmette-Guerin (BCG)	Anti-PD-1	Non-Muscle-Invasive Bladder cancer (Biologic)	Phase 3	New Molecular Entity

- Regulatory Designations – See Definitions in Backup
- Neulasta® is a registered U.S. trademark of Amgen Inc.
- Rituxan® is a registered U.S. trademark of Biogen MA Inc.; MabThera® is a trademark of F. Hoffmann La Roche AG
- Erbitux® is a registered trademark of ImClone LLC

Oncology (2 of 3)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
Ibrance (palbociclib)	CDK 4,6 kinase inhibitor	ER+/HER2+ Breast Cancer	Phase 3	Product Enhancement
Lorbrena (lorlatinib)	ALK inhibitor	1st Line ALK Non-Small Cell Lung Cancer (ORPHAN - U.S.)	Phase 3	Product Enhancement
Talzenna (talazoparib)	PARP inhibitor	Combo w/ Xtandi (enzalutamide) for 1st Line Metastatic Castration-Resistant Prostate Cancer	Phase 3	Product Enhancement
Xtandi (enzalutamide)	Androgen receptor inhibitor	Non-metastatic High Risk Hormone Sensitive Prostate Cancer	Phase 3	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	1st Line Merkel Cell Carcinoma (MCC) (Biologic)	Phase 2	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Combo w/CMP-001 for Head and Neck Cancer	Phase 2	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Combo w/Talzenna (talazoparib) for Locally Advanced (Primary or Recurrent) or Metastatic Solid Tumors (Biologic)	Phase 2	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Combo w/Talzenna (talazoparib) for Solid Tumors with a BRCA or ATM defect (Biologic)	Phase 2	Product Enhancement
Braftovi (encorafinib) + Mektovi (binimetinib)	*BRAF* kinase inhibitor and MEK inhibitor	BRAF-mutant Metastatic Melanoma (ORPHAN - U.S.)	Phase 2	Product Enhancement
Braftovi (encorafinib) + Mektovi (binimetinib)	*BRAF* kinase inhibitor and MEK inhibitor	1st line BRAF-mutant Colorectal Cancer	Phase 2	Product Enhancement
Braftovi (encorafinib) + Mektovi (binimetinib)	*BRAF* kinase inhibitor and MEK inhibitor	1st line BRAF-mutant Non-Small Cell Lung Cancer	Phase 2	Product Enhancement
Daurismo (glasdegib)	SMO (smoothened) antagonist	Myelodysplastic Syndrome	Phase 2	Product Enhancement

- Regulatory Designations – See Definitions in Backup

Oncology (3 of 3)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
Talzenna (talazoparib)	PARP inhibitor	2nd Line Metastatic Castration-Resistant Prostate Cancer	Phase 2	Product Enhancement
Talzenna (talazoparib)	PARP inhibitor	Germline BRCA Mutated Locally Advanced Triple Negative Breast Cancer	Phase 2	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Combo w/Talzenna (talazoparib) and binimetinib for Solid Tumors (Biologic)	Phase 1	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Cancer (Biologic)	Phase 1	Product Enhancement
PF-05082566	CD137 Agonist	Combo w/Kite Pharma's Yescarta® (axicabtagene ciloleucel) for Cancer	Phase 1	New Molecular Entity
PF-06647020	protein tyrosine kinase 7 (PTK7) Targeted Cytotoxicity	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-06804103	HER2 Antibody Drug Conjugate	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-06821497	EZH2 inhibitor	Cancer	Phase 1	New Molecular Entity
PF-06863135	BCMA-CD3 Bispecific Antibody	Multiple Myeloma (Biologic)	Phase 1	New Molecular Entity
PF-06873600	CDK 2,4,6 inhibitor	Breast Cancer Metastatic	Phase 1	New Molecular Entity
PF-06952229	transforming growth factor, beta receptor 1 (TGFBR1) Inhibitor	Cancer	Phase 1	New Molecular Entity
PF-06939999	protein arginine methyltransferase 5 (PRMT5) Inhibitor	Solid Tumors	Phase 1	New Molecular Entity
▶ PF-07062119	GUCY2c CD3 Bispecific Antibody	Solid Tumors (Biologic)	Phase 1	New Molecular Entity
▶ PF-06940434	Integrin alpha-V/beta-8 Antagonist	Solid Tumors (Biologic)	Phase 1	New Molecular Entity
PF-06753512	Therapeutic Vaccine	Prostate Cancer	Phase 1	New Molecular Entity
PF-06936308	Therapeutic Vaccine	Multiple Cancers	Phase 1	New Molecular Entity

▶ Indicates that the project is either new or has progressed in phase since the previous portfolio update of Pfizer.com

- PF-06753512 Prostate Cancer and PF-06936308 Multiple Cancers have been moved to Oncology from Vaccines
- Yescarta® is a registered U.S. trademark of Kite Pharma, Inc.

Rare Diseases

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
Vyndaqel (tafamidis meglumine)	Transthyretin (TTR) Dissociation Inhibitor	Transthyretin familial amyloid polyneuropathy (U.S.) (FAST TRACK, ORPHAN - U.S.)	Registration	New Molecular Entity
Vyndaqel (tafamidis meglumine and free acid)	Transthyretin (TTR) Dissociation Inhibitor	Transthyretin Amyloid Cardiomyopathy (E.U.) (ORPHAN)	Registration	Product Enhancement
PF-07265803	p38 Mitogen-Activated Protein Kinase	Dilated Cardiomyopathy due To Lamin A/C Gene Mutation	Phase 3	New Molecular Entity
fidanacogene elaparvovec (PF-06838435)	Gene Therapy, coagulation factor IX (F9)	Hemophilia (Biologic) (BREAKTHROUGH, ORPHAN - U.S., E.U., PRIME - E.U.)	Phase 3	New Molecular Entity
somatrogon (PF-06836922)	Human Growth Hormone Agonist	Pediatric Growth Hormone Deficiency (Biologic) (ORPHAN - U.S., E.U.)	Phase 3	New Molecular Entity
somatrogon (PF-06836922)	Human Growth Hormone Agonist	Adult Growth Hormone Deficiency (Biologic) (ORPHAN - U.S., E.U.)	Phase 3	Product Enhancement
rivipansel (GMI-1070)	Pan-Selectin Antagonist	Acute vaso-occlusive crises associated with sickle cell disease in patients aged 6 years and above (FAST TRACK, ORPHAN - U.S., E.U.)	Phase 3	New Molecular Entity
PF-07055480 (SB-525)	AAV-FVIII GTx	Hemophilia (Biologic) (RMAT, FAST TRACK, ORPHAN - U.S.; ORPHAN – E.U.)[1]	Phase 2	New Molecular Entity
PF-06730512	SLIT2 antagonist	Focal Segmental Glomerulosclerosis (FSGS) (Biologic)	Phase 2	New Molecular Entity
marstacimab (PF-06741086)	Tissue Factor Pathway Inhibitor (TFPI)	Hemophilia (Biologic) (FAST TRACK – U.S.; ORPHAN - U.S., E.U.)	Phase 2	New Molecular Entity
PF-05230907	Factor Xa Protein Replacement	Intracerebral Hemorrhage (Biologic) (ORPHAN - U.S.)	Phase 1	New Molecular Entity
PF-06755347	Immunomodulation	Chronic Inflammatory Demyelination Polyneuropathy	Phase 1	New Molecular Entity
PF-06939926	minidystrophin	Duchenne Muscular Dystrophy (Biologic) (ORPHAN - U.S., E.U.)	Phase 1	New Molecular Entity
recifercept	Soluble recombinant human fibroblast growth factor receptor 3 (FGFR3) decoy	Achondroplasia (Biologic)	Phase 1	New Molecular Entity

►Indicates that the project is either new or has progressed in phase since the previous portfolio update of Pfizer.com
1 - Lead-in trial of the Phase 3 clinical program ongoing
• Regulatory Designations – See Definitions in Backup

Vaccines

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
PF-06425090	Prophylactic Vaccine	Primary *clostridioides difficile* infection (FAST TRACK)	Phase 3	New Molecular Entity
PF-06482077	Prophylactic Vaccine	Invasive and Non-Invasive Pneumococcal infections (Adult) (BREAKTHROUGH)	Phase 3	New Molecular Entity
PF-06842433	Prophylactic Vaccine	Invasive and Non-Invasive Pneumococcal infections	Phase 2	New Molecular Entity
PF-06760805	Prophylactic Vaccine	Invasive Group B Streptococcus Infection	Phase 2	New Molecular Entity
PF-06886992	Prophylactic Vaccine	Serogroups ABCWY Meningococcal Infections	Phase 2	New Molecular Entity
PF-06928316	Prophylactic Vaccine	Respiratory Syncytial Virus Infection	Phase 2	New Molecular Entity

- PF-06753512 Prostate Cancer and PF-06936308 Multiple Cancers have been moved to Oncology
- Regulatory Designations – See Definitions in Backup

Hospital (Anti-Infectives)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
aztreonam-avibactam (PF-06947387)	Beta Lactam/Beta Lactamase Inhibitor	Treatment of infections caused by Gram-negative bacteria for which there are limited or no treatment options	Phase 3	New Molecular Entity

Programs Discontinued from Development since October 29, 2019

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
Bavencio (avelumab)	Anti PD-L1	1st Line Gastric Cancer (Biologic)	Phase 3	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Combo w/PF-04518600 (OX40) for various Solid Tumors (Biologic)	Phase 2	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	Combo w/PF-05082566 (anti-4-1BB/CD137) for various Solid Tumors (Biologic)	Phase 2	Product Enhancement
Inlyta (axitinib)	VEGFR tyrosine kinase inhibitor	Combo w/Merck's Keytruda® (PD-1, pembrolizumab) for Cancer	Phase 1	Product Enhancement
PF-06688992	Antibody Drug Conjugate	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-04447943	PDE9 Inhibitor	Sickle Cell Anemia (ORPHAN - U.S.)	Phase 1	New Molecular Entity
Bavencio (avelumab)	Anti PD-L1	Combo w/PF-04518600 (OX40) and PF-05082566 (anti-4-1BB/CD137) for Cancer (Biologic)	Phase 1	Product Enhancement

- Keytruda® is a registered U.S. trademark of Merck Sharp & Dohme Corp.

Backup

Regulatory Designation Definitions

- **Fast Track** (U.S.) is a designation available to a product if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. This designation is intended to facilitate development and expedite review of drugs to treat serious and life-threatening conditions so that an approved product can reach the market expeditiously. More information about the qualifying criteria and features of the Fast Track program can be found on the FDA's website.
- **Breakthrough Designation** (U.S.) may be granted to a drug (alone or in combination with 1 or more other drugs) intended to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A drug that receives breakthrough designation is eligible for all fast track designation features and an FDA commitment to work closely with the sponsor to ensure an efficient drug development program. More information about the qualifying criteria and features of the Breakthrough program can be found on the FDA's website.
- **Orphan Drug** (U.S.) - Orphan drug status may be granted to drugs and biologics that are intended for the diagnosis, prevention, or treatment of rare diseases/disorders that affect fewer than 200,000 people in the U.S., or that affect more than 200,000 persons but where it is unlikely that expected sales of the product would cover the sponsor's investment in its development. More information about the qualifying criteria, features, and incentives involved in an orphan drug designation can be found on the FDA's website.
- **Orphan Drug** (E.U.) - Orphan drug status may be granted to drugs and biologics that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 persons in the European Union at the time of submission of the designation application, or that affect more than 5 in 10,000 persons but where it is unlikely that expected sales of the product would cover the investment in its development. More information about the qualifying criteria, features, and incentives involved in an orphan drug designation can be found on the EMA's website.
- A U.S. drug application will receive a **priority review designation** if it is for a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. A priority designation is intended to direct overall attention and resources to the evaluation of such applications. A priority review designation means that FDA's goal is to take action on the marketing application within 6 months of receipt (compared with 10 months under standard review). More information about the qualifying criteria and features of a priority review designation can be found on the FDA's website.
- **PRIME** (E.U.) **-** The PRIME scheme is applicable to products under development which are innovative and yet to be placed on the EU market. The scheme aims to support medicinal products of major public health interest and in particular from the viewpoint of therapeutic innovation. Medicines eligible for PRIME must address an unmet medical need, i.e. for which there exists no satisfactory method of diagnosis, prevention or treatment in the Community or, if such a method exists, in relation to which the medicinal product concerned will be of major therapeutic advantage to those affected. A product eligible for PRIME should demonstrate the potential to address, to a significant extent, the unmet medical need, for example by introducing new methods of therapy or improving existing ones. Data available to support the request for eligibility should support the claim to address the unmet medical need through a clinically meaningful improvement of efficacy, such as having an impact on the prevention, onset or duration of the condition, or improving the morbidity or mortality of the disease. EMA will provide early and enhanced support to optimize the development of eligible medicines. Products granted PRIME support are anticipated to benefit from the Accelerated Assessment procedure. More information about the qualifying criteria and features of PRIME and Accelerated Assessment can be found on the EMA's website.
- **Regenerative Medicine Advanced Therapy (RMAT)** (U.S.) is a designation that is granted to regenerative medicine therapies intended to treat, modify, reverse, or cure a serious condition, for which preliminary clinical evidence indicates that the medicine has the potential to address an unmet medical need. The RMAT designation includes all the benefits of the fast track and breakthrough therapy designation programs, including early interactions with FDA.