



# Pfizer Pipeline

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As of July 29, 2019

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# 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 July 29, 2019.
- Visit [Pfizer.com/pipeline](https://www.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.

# Table of Contents

<b>Pfizer Pipeline Snapshot</b>	<b>4</b>
<b>Inflammation and Immunology</b>	<b>5-6</b>
<b>Internal Medicine</b>	<b>7</b>
<b>Oncology</b>	<b>8-11</b>
<b>Rare Diseases</b>	<b>12</b>
<b>Vaccines</b>	<b>13</b>
<b>Hospital (Anti-Infectives)</b>	<b>14</b>
<b>Backup: Regulatory Designation Definitions</b>	<b>15-16</b>

# Pfizer Pipeline Snapshot



**Pfizer Pipeline Snapshot as of July 29, 2019**

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

- 8 programs advanced or are new
- Included are 54 NMEs, 44 additional indications, plus 3 biosimilars

### Recent Approvals

- BAVENCIO® (avelumab) in combination with INLYTA (axitinib) for the first-line treatment of patients with advanced renal cell carcinoma (U.S.)
- LORVIQUA® (lorlatinib) for the treatment of adult patients with ALK- positive advanced non-small cell lung cancer whose disease has progressed after alectinib or ceritinib as the first ALK tyrosine kinase inhibitor (TKI) therapy; or crizotinib and at least one other ALK TKI (E.U.)
- RUXIENCE™, a biosimilar to Rituxan<sup>(1)</sup> (rituximab), for the treatment of adult patients with non-Hodgkin's lymphoma, chronic lymphocytic leukemia, and granulomatosis with polyangiitis and microscopic polyangiitis (U.S.)
- TALZENNA® (talazoparib) for the treatment of adult patients with germline breast cancer susceptibility gene (gBRCA)1/2-mutations, who have human epidermal growth factor receptor 2-negative (HER2-) locally advanced or metastatic breast cancer (E.U.)
- VYNDAQEL® (tafamidis meglumine) and VYNDAMAX™ (tafamidis) for the treatment of the cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization (U.S.)
- ZIRABEV™, a biosimilar to Avastin<sup>(2)</sup> (bevacizumab), for the treatment of treatment of metastatic colorectal cancer; unresectable, locally advanced, recurrent or metastatic NSCLC; recurrent glioblastoma; metastatic RCC; and persistent, recurrent or metastatic cervical cancer (U.S.)



**Pfizer Pipeline Snapshot as of April 30, 2019**

Pipeline represents progress of R&D programs as of April 30, 2019

- 5 programs advanced or are new
- 1 program discontinued since last update
- Included are 54 NMEs, 39 additional indications, plus 4 biosimilars

### Recent Approvals

- TRAZIMERA™ (trastuzumab-qyyp), a biosimilar to Herceptin<sup>(3)</sup> (trastuzumab), for the treatment of human epidermal growth factor receptor-2 (HER2) overexpressing breast cancer and HER2 overexpressing metastatic gastric or gastroesophageal junction adenocarcinoma (U.S.)
- VIZIMPRO® (dacomitinib) for the first-line treatment of adult patients with locally advanced or metastatic non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR)-activating mutations (E.U.)
- ZIRABEV™<sup>(2)</sup>, a biosimilar to Avastin® (bevacizumab), for the treatment of metastatic carcinoma of the colon or rectum, metastatic breast cancer, unresectable advanced, metastatic or recurrent non-small cell lung cancer (NSCLC), advanced and/or metastatic renal cell cancer and persistent, recurrent or metastatic carcinoma of the cervix (E.U.)



(1) Rituxan® is a registered U.S. trademark of Biogen MA Inc.; MabThera® is a trademark of F. Hoffmann La Roche AG  
 (2) Avastin® is a registered U.S. trademark of Genentech, Inc.  
 (3) Herceptin® is a registered U.S. trademarks of Genentech, Inc

# Inflammation and Immunology (1 of 2)



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) (U.S.) / (E.U.)	Registration	Biosimilar
Xeljanz (tofacitinib)	JAK Inhibitor	Modified Release 11mg Tablet for Rheumatoid Arthritis (E.U.)	Registration	Product Enhancement
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	Inflammatory Bowel Disease (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-06651600	JAK3/TEC	Ulcerative Colitis	Phase 2	Product Enhancement
PF-06651600	JAK3/TEC	Crohn's Disease	Phase 2	Product Enhancement
PF-06651600	JAK3/TEC	Vitiligo	Phase 2	Product Enhancement
PF-06700841	TYK2/JAK1	Alopecia Areata	Phase 2	New Molecular Entity
PF-06700841	TYK2/JAK1	Psoriasis	Phase 2	Product Enhancement
PF-06700841	TYK2/JAK1	Ulcerative Colitis	Phase 2	Product Enhancement
PF-06700841	TYK2/JAK1	Crohn's Disease	Phase 2	Product Enhancement
PF-06700841	TYK2/JAK1	Vitiligo	Phase 2	Product Enhancement



- 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	Psoriatic Arthritis	Phase 2	Product Enhancement
▶ PF-06700841	TYK2/JAK1	Lupus	Phase 2	Product Enhancement
▶ PF-06700841	Topical TYK2/JAK1	Atopic Dermatitis	Phase 2	Product Enhancement
▶ PF-06700841	Topical TYK2/JAK1	Psoriasis	Phase 2	Product Enhancement
▶ PF-06826647	TYK2 Inhibitor	Psoriasis	Phase 2	New Molecular Entity
PF-06823859	interferon, beta 1, fibroblast (IFNB1) Blocker	Inflammatory Disorders (Biologic)	Phase 2	New Molecular Entity
PF-06763809	Transcription Factor Inhibitor	Psoriasis	Phase 1	New Molecular Entity
PF-06817024	Cytokine Modulator	Atopic Dermatitis (Biologic)	Phase 1	New Molecular Entity
PF-06826647	TYK2 Inhibitor	Inflammatory Bowel Disease	Phase 1	New Molecular Entity
PF-06835375	CXCR5 Antagonist	Lupus (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
- Regulatory Designations – See Definitions in Backup



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-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	Phase 1	New Molecular Entity
PF-06946860	Growth Factor Blocker	Cachexia (Biologic)	Phase 1	New Molecular Entity

# Oncology (1 of 4)



Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
PF-05280586, a potential biosimilar to Rituxan® /MabThera® (rituximab)	CD20 antigen antagonist	Follicular Lymphoma (Biosimilar) (E.U.)	Registration	Biosimilar
Bavencio (avelumab)	Anti PD-L1	1st Line Renal Cell Carcinoma (Biologic) (Combo w/ Inlyta (axitinib)) (E.U.)	Registration	Product Enhancement
► 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
Bavencio (avelumab)	Anti PD-L1	1st Line Non-Small Cell Lung Cancer (Biologic)	Phase 3	Product Enhancement
Bavencio (avelumab)	Anti PD-L1	1st Line Gastric 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



- 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
- Rituxan® is a registered U.S. trademark of Biogen MA Inc.; MabThera® is a trademark of F. Hoffmann La Roche AG



# Oncology (2 of 4)

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	1 <sup>st</sup> 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	Metastatic Hormone Sensitive Prostate Cancer (U.S.)	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/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
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
Daurismo (glasdegib)	SMO (smoothened) antagonist	Myelodysplastic Syndrome	Phase 2	Product Enhancement



# Oncology (3 of 4)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
Talzenna (talazoparib)	PARP inhibitor	2 <sup>nd</sup> 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/PF-04518600 (OX40) and PF-05082566 (anti-4-1BB/CD137) for Cancer (Biologic)	Phase 1	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
gedatolisib (PF-05212384)	pan-PI3K/mTOR inhibitor	Cancer	Phase 1	New Molecular Entity
Ibrance (palbociclib)	CDK 4,6 kinase inhibitor	Combo w/gedatolisib (PF-05212384) for Cancer	Phase 1	Product Enhancement
Inlyta (axitinib)	VEGFR tyrosine kinase inhibitor	Combo w/Merck's Keytruda® (PD-1, pembrolizumab) for Cancer	Phase 1	Product Enhancement
PF-04518600	OX40 receptor Agonist	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-06647020	protein tyrosine kinase 7 (PTK7) Targeted Cytotoxicity	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-06671008	cadherin 3, type 1, P-cadherin (placental) (CDH3)	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-06688992	Antibody Drug Conjugate	Cancer (Biologic)	Phase 1	New Molecular Entity
PF-06801591	Anti-PD-1	Cancer Immunotherapy (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



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

# Oncology (4 of 4)

Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
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-06881894, a potential biosimilar to Neulasta® (Pegfilgrastim)	Human Granulocyte Colony Stimulating Factor	Neutropenia in patients undergoing cancer chemotherapy (Biosimilar)	Phase 1	Biosimilar
PF-06952229	transforming growth factor, beta receptor 1 (TGFB1) Inhibitor	Cancer	Phase 1	New Molecular Entity
PF-06939999	protein arginine methyltransferase 5 (PRMT5) Inhibitor	Solid Tumors	Phase 1	New Molecular Entity



- Neulasta® is a registered U.S. trademark of Amgen 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
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
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
somatrogon (PF-06836922)	Human Growth Hormone Agonist	Adult Growth Hormone Deficiency (Biologic) (ORPHAN - U.S., 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	Product Enhancement
PF-06730512	SLIT2 antagonist	Focal Segmental Glomerulosclerosis (FSGS) (Biologic)	Phase 2	New Molecular Entity
PF-06741086	Tissue Factor Pathway Inhibitor (TFPI)	Hemophilia (Biologic) (ORPHAN - U.S., E.U.)	Phase 2	New Molecular Entity
PF-07055480 (SB-525)	AAV-FVIII GTx	Hemophilia (Biologic) (RMAT, FAST TRACK, ORPHAN - U.S.; ORPHAN - E.U.)	Phase 2	New Molecular Entity
PF-04447943	PDE9 Inhibitor	Sickle Cell Anemia (ORPHAN - U.S.)	Phase 1	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

- Regulatory Designations – See Definitions in Backup

# Vaccines



Compound Name	Mechanism of Action	Indication	Phase of Development	Submission Type
PF-06425090	Prophylactic Vaccine	Primary <i>clostridium difficile</i> infection (FAST TRACK)	Phase 3	New Molecular Entity
PF-06482077	Prophylactic Vaccine	Invasive and non-invasive Pneumococcal infections (BREAKTHROUGH)	Phase 3	New Molecular Entity
PF-06842433	Prophylactic Vaccine	Invasive and non-invasive Pneumococcal infections	Phase 2	New Molecular Entity
PF-06753512	Therapeutic Vaccine	Prostate Cancer	Phase 1	New Molecular Entity
PF-06760805	Prophylactic Vaccine	Invasive Group B streptococcus infection	Phase 1	New Molecular Entity
PF-06886992	Prophylactic Vaccine	Serogroups ABCWY meningococcal infections	Phase 1	New Molecular Entity
PF-06928316	Prophylactic Vaccine	Respiratory Syncytial Virus Infection	Phase 1	New Molecular Entity
PF-06936308	Therapeutic Vaccine	Multiple Cancers	Phase 1	New Molecular Entity



• 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



# 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.