# 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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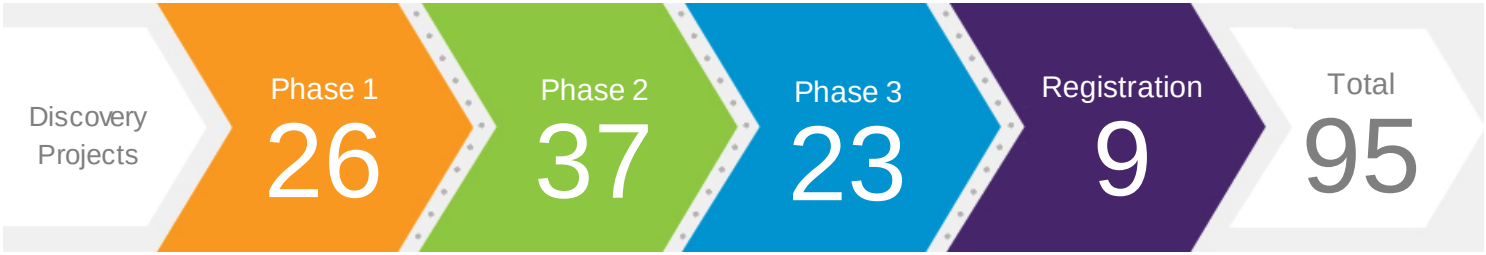
# Pfizer Pipeline Snapshot



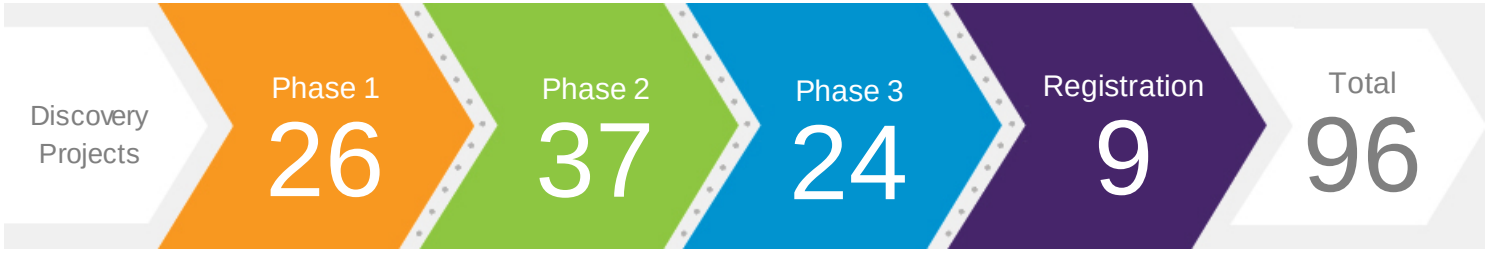

**Pfizer Pipeline Snapshot as of January 28, 2020**

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<br>PF-06700841<br>PF-06826647 | IRAK4<br>TYK2/JAK1<br>TYK2 Inhibitor | Hidradenitis Suppurativa | Phase 2 | Product Enhancement |
| PF-06651600<br>PF-06700841 | JAK3/TEC<br>TYK2/JAK1 | Ulcerative Colitis | Phase 2 | New Molecular Entity |
| PF-06651600<br>PF-06700841 | JAK3/TEC<br>TYK2/JAK1 | Crohn's Disease | Phase 2 | Product Enhancement |
| PF-06651600<br>PF-06700841 | JAK3/TEC<br>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 | CXCR5 Antagonist | 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 |

▶ 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

# 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.