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# 1ST QUARTER 2021
# PIPELINE REPORT

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## PIPELINE HIGHLIGHTS:

### Recent Approvals

- **Zokivny (lonafarnib)** – Premature aging disease: progeria. FDA approval: 11/20/2020
- **Oxlumo (lumasiran; aka ALN-GO1)** – Kidney disease: primary hyperoxaluria type 1. FDA approval: 11/23/2020
- **Danyelza (naxitamab)** – Nervous system cancer: Neuroblastoma. FDA approval: 11/25/2020
- **Imcivree (setmelanotide; aka RM-493)** – Obesity: due to genetic defects. FDA approval: 11/27/2020
- **Orladeyo (berotralstat; aka bcx7353)** – Hereditary Angioedema (HAE). FDA approval: 12/3/2020

### Anticipated FDA Approvals

- **dostarlimab (aka TSR-042)** - Cancer: endometrial. Expected FDA decision: 4Q2020
- **umbralisib (aka TGR-1202)** - Cancer: Marginal zone lymphoma. Expected FDA decision: 2/15/2021
- **trilaciclib** - Prevention of chemotherapy-induced immune suppression. Expected FDA decision: 2/15/2021
- **Amondys 45 (casimersen)** - Duchenne muscular dystrophy. Expected FDA decision: 2/25/2021
- **Ygalo (melflufen)** - Blood cancer: multiple myeloma. Expected FDA decision: 2/28/2021
- **ropeginterferon alfa-2b** - Blood cancer: Polycythaemia vera. Expected FDA decision: 2/2021
- **tepotinib** - Lung cancer. Expected FDA decision: 2/2021
- **aducanumab (aka BIIB037)** - Alzheimer's disease. Expected FDA decision: 3/7/2021
- **arimoclomol** - Genetic disorder: Niemann-Pick disease type C. Expected FDA decision: 3/17/2021
- **idecabtagene vicleucel (aka ide-cel)** - Blood cancer: multiple myeloma. Expected FDA decision: 3/27/2021
- **Fotivda (tivozanib)** - Kidney cancer. Expected FDA decision: 3/31/2021
- **fosdenopterin** - Genetic disorder: Molybdenum cofactor deficiency. Expected FDA decision: 3/2021
- **ponesimod (aka ACT 128800)** - Multiple sclerosis. Expected FDA decision: 1Q2021

## Recent FDA Approvals

| Approval Date | Estimated WAC / Year & Benefit coverage | Drug Name / Manufacturer | Treatment Indication | Disease or Administration Comments | Clinical Comments | Therapy Options Approximate WAC/Year |
|---|---|---|---|---|---|---|
| 11/20/2020 | **Projected: $650,000** (launch in January 2021)<br><br>Co-payment assistance, reimbursement support, patient support, and patient assistance programs may be available through the Eiger OneCare Program<br><br>**Pharmacy benefit** | **Zokinvy** lonafarnib<br><br>**Eiger BioPharmaceuticals**<br>**Merck & Co** | **Accelerated aging conditions**: To reduce risk of mortality in Hutchinson-Gilford progeria syndrome and treatment of progeroid laminopathies | Oral<br><br>An estimated 400 children worldwide have progeria and progeroid laminopathies.<br><br>**FDA Designations**: Orphan Drug, Breakthrough Therapy, Priority Review, Rare Pediatric Disease | Farnesyltransferase inhibitor: lonafarib prevents a step in production of abnormal proteins otherwise produced by mutated genes.<br><br>Historically, children with progeria lived to an average age of 14.5 years.<br><br>Due to the dearth of patients overall, utilization of this product will be low. Also, this means that results from clinical trials are not necessarily reliable. However, lack of alternatives, combined with severity of these conditions, portend good adoption. | **Therapy alternatives**<br>None |
| 11/23/2020 | **Projected: $490,000** (launch in 4Q2020)<br><br>Manufacturer will reportedly offer value-based agreements (VBAs) to payers with an additional Patient Needs Adjustment (PNA) rebate to account for weight-based dosing.<br><br>**Pharmacy Benefit** | **Oxlumo** lumasiran (aka ALN-GO1)<br><br>**Alnylam Dicerna Pharmaceuticals** | **Kidney disease**: Primary hyperoxaluria type 1 (PH1) | Subcutaneous<br><br>PH1 is an ultra-rare, inherited disorder resulting from a buildup of oxalate, which combines with calcium to form kidney and bladder stones. It can lead to end-stage renal disease, which may be life threatening. 1-3 million people in North America have PH1.<br><br>**FDA Designations**: Priority Review, Orphan Drug, Breakthrough Therapy, and Pediatric Rare Disease | Prevents production of oxalate by blocking an enzyme.<br><br>Another product less advanced in development has potential to overshadow lumasiran with better dosing and broader indications. At this time, however, this product is likely to gain market share due to a lack of competition. | **Therapy alternatives**<br>None |

## Recent FDA Approvals *(continued)*

| Approval Date | Estimated WAC / Year & Benefit coverage | Drug Name / Manufacturer | Treatment Indication | Disease or Administration Comments | Clinical Comments | Therapy Options Approximate WAC/Year |
|---|---|---|---|---|---|---|
| 11/25/2020 | **$2,977,650**<br><br>Copayment assistance may be available through the Y-mAbs Connect Co-pay Program.<br><br>**Medical benefit** | **Danyelza**<br>naxitamab-gqgk<br><br>**Y-mAbs Therapeutics** | **Nervous system cancer**: Relapsed or refractory, high risk neuroblastoma in bone or bone marrow | IV infusion<br><br>Neuroblastoma occurs most often in infants and children Often starts in nerve tissue in the abdomen; may behave in many different ways. Diagnosed in about 700 children in the US each year.<br><br>**FDA designation**: Priority review, Breakthrough Therapy, Orphan Drug, Rare Pediatric Disease | **Biologic product**<br>Antibody that binds to tumor cells, triggering a response that kills tumor cells. New mechanism of action for treating this condition.<br><br>Very few alternatives exist for this form of cancer at this stage. Given efficacy and similarity of cost with best alternative, Danyelza may become a first choice. | **Therapy alternatives**<br>> Chemotherapy plus biologic therapy: $2.3Million<br>> Clinical trials (cost varies) |
| 11/27/2020 | **Projected $240,900**<br>(launch in 1Q 2021)<br><br>**Pharmacy benefit** | **Imcivree**<br>setmelanotide (aka RM-493)<br><br>**Rhythm Pharmaceuticals** | **Obesity**: severe obesity and insatiable hunger that result from rare genetic defects in the MC4R pathway | Subcutaneous<br><br>These conditions are ultra-rare, with an estimated prevalence of <1 in 1,000,000.<br><br>**FDA designation**: Breakthrough Therapy, Orphan Drug | Melanocyte-stimulating hormone analog: restores activation of the MC4R pathway in the hypothalamus.<br><br>Due to the dearth of patients overall, utilization of this product will be low. Also, this means that results from clinical trials are not necessarily reliable. However, lack of alternatives, combined with severity of this condition, portend good adoption for patients with these conditions. | **Therapy alternatives**<br>None |
| 12/3/2020 | **$485,000**<br><br>Patient support is available through Empower Patient Services<br><br>**Pharmacy benefit** | **Orladeyo**<br>berotralstat (aka bcx7353)<br><br>**BioCryst** | **Hereditary Angioedema (HAE)**: Prevention of hereditary angioedema (HAE) attacks | Oral<br><br>HAE, which affects about 1 in 50,000 people, causes recurrent episodes of severe swelling in the limbs, face, intestinal tract, and airway. Swelling in the airway can cause life-threatening airway obstruction.<br><br>Untreated HAE patients have, on average, a 3-4 day attack every 1 to 2 weeks. | Plasma kallikrein inhibitor: inhibits uncontrolled activity of kallikrein, which otherwise causes overproduction of symptom-causing bradykinin. It is the first non-androgen oral therapy for the prevention of HAE attacks.<br><br>Does not appear to be as effective as competitor product Takhzyro, may not have high uptake given similar pricing. | **Therapy alternatives**<br>> Takhzyro $591,035<br>> Cinryze $573,828<br>> Haegarda $503,464 |

## Anticipated FDA Approvals

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 4Q2020 | **Brand Name TBD**<br>dostarlimab (aka TSR-042)<br><br>**Tesaro GSK**<br>**AnaptysBio** | **Endometrial cancer**: Recurrent or advanced mismatch repair-deficient (dMMR) endometrial cancer which progressed on or after a platinum-based regimen. | Injectable<br><br>Endometrial cancer is the most common cancer of the female reproductive organs, with over 65,000 new cases and 12,600 deaths expected for 2020. dMMR is found in up to 30% of recurrent endometrial cancer.<br><br>Mismatch repair mechanisms correct mistakes made within cells when DNA is copied. Cells that are mismatch repair deficient usually have many DNA mutations, some that can cause cancer. | **Biologic product**<br>Programmed cell death 1 (PD-1) inhibitor: a "checkpoint inhibitor" that prevents interruption of an immune response that targets cancer cells.<br><br>If approved, would be the 7th drug in this class. Other products include Libtayo (cemiplimab), Opdivo (nivolumab) and Keytruda (pembrolizumab). These are used for many types of cancer. | **Therapy alternatives**<br>Pembrolizumab |
| 2/15/2021 | **Brand Name TBD**<br>umbralisib<br>(aka TGR-1202)<br><br>**TG Therapeutics** | **Lymphoma (cancer)**: Marginal zone lymphoma (MZL) | Oral<br><br>MZL is a slow-growing type of non-Hodgkin lymphoma, comprising about 8% of the 75,000+ cases of non-Hodgkin lymphoma diagnosed annually in the US.<br><br>**FDA designations**: Breakthrough Therapy, Priority Review | Phosphoinositide 3-kinase (PI3K) and casein kinase-1 epsilon inhibitor<br><br>There are at least 4 other PI3K inhibitors on the market, used to treat lymphomas, leukemias and breast cancer. | **Therapy alternatives**<br>Combination regimen such as<br>> "R-CHOP"<br>OR<br>> Bendamustine + obinutuzumab |
| 2/15/2021 | **Brand Name TBD**<br>trilaciclib<br><br>**G1 Therapeutics**<br>**Boehringer Ingelheim** | **Prevention of chemotherapy-associated immune suppression**: in patients with small cell lung cancer being treated with chemotherapy | Intravenous<br><br>Treatment for SCLC is particularly hard on the bone marrow, and can cause severe drops in red blood cells, white blood cells and/or platelets. These effects often result in the need to reduce a patient's chemo dose.<br><br>**FDA designations**: Breakthrough therapy; Priority Review | CDK4/6 dual inhibitor--protects bone marrow cells that produce red blood cells, white blood cells and platelets from chemotherapy toxicity.<br><br>Is unique in that it is given before toxicity begins.<br><br>Also being tested in patients being treated for colorectal and breast cancer. | **Therapy alternatives**<br>> Filgrastim (e.g., Neupogen)<br>> Pegfilgrastim (e.g., Neulasta)<br>> Sargramostim (Leukine) |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 02/25/2021 | **Amondys 45**<br>casimersen<br><br>**Sarepta Therapeutics** | **Duchenne Muscular Dystrophy** (DMD) in patients with mutation amenable to exon 45 skipping | IV<br><br>DMD is a fatal genetic neuromuscular disorder affecting one in 3,500 - 5,000 males worldwide.<br><br>DMD patients with mutations amenable to exon 45 skipping represent about 8% of DMD patients.<br><br>**FDA designations**: Orphan Drug, Priority Review | Masks the mutation in the DMD gene, allowing production of a functional protein in place of the one causing DMD symptoms.<br><br>Amondys 45 is the first product specific to exon 45 skipping. | **Therapy alternatives**<br>None |
| 02/28/2021 | **Ygalo**<br>melflufen<br><br>**Oncopeptides** | **Blood cancer**:<br>Multiple myeloma, relapsed or refractory | IV<br><br>Second most common form of blood cancer in the US, it is currently treatable to a certain extent but not curable. Median age at diagnosis is 69 years.<br><br>In 2020:<br>> About 32,270 new cases will be diagnosed<br><br>> About 12,830 deaths are expected to occur<br><br>**FDA designations**: Priority Review | Alkylating agent: Kills cells that are similar to (and include) myeloma cells. | **Therapy alternatives**<br>> Triple combinations that include Darzalex (daratumumab) with dexamethasone and one of the following:<br>> Velcade (bortezomib) OR<br>> Kyprolis (carfilzomib) OR<br>> Ienalidamide<br><br>> Blenrep (belantamab mafodotin-blmf) |
| 02/2021 | **Brand Name TBD**<br>ropeginterferon alfa-2b<br><br>**PharmaEssentia** | **Blood cancer**:<br>Polycythaemia Vera (PV) | Subcutaneous, every 2 weeks<br><br>PV results in too many red blood cells in the blood, thickening it and causing serious effects such as blood clots.<br><br>PV develops slowly over a number of years. It is rare, affecting approximately 2 people per 100,000. It occurs most often in individuals older than 60 years.<br><br>**FDA designations**: Orphan drug | **Biologic product**<br>Pegylated interferons<br><br>It appears that the effects of ropeginterferon build more slowly than those of standard therapy, and may last longer.<br><br>Ropeginterferon alfa-2b was approved by the European Commission in February 2019. | **Therapy alternatives**<br>Hydroxyurea |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 02/2021 | **Brand name TBD**<br>tepotinib<br><br>**Merck, KGaA, EMD Serono** | **Lung cancer:**<br>Metastatic non-small cell lung cancer (NSCLC) in tumors with MET gene alterations | Oral<br><br>In the US in 2020, there were approximately 228,000 new cases of lung cancer. MET gene alterations are found in 3% to 5% of NSCLC cases.<br><br>**FDA Designation:** Priority Review, Real-Time Oncology Review pilot | C-MET inhibitor: inhibits signaling from dysfunctional MET receptors, which causes cancer. | **Therapy alternatives**<br>> Xalkori (crizotinib)<br>> Tabrecta (capmatinib) |
| 03/07/2021 | **Brand Name TBD**<br>aducanumab<br>(aka BIIB037)<br><br>**Biogen Neurimmune** | **Alzheimer's disease (AD)** | Intravenous<br><br>AD is expected to affect 13.9 million Americans or 3.3% of the country's population, by 2060.<br><br>**FDA designations:**<br>Priority review<br><br>As of 11/6, the FDA Advisory panel did not recommend approval of this product because "the evidence wasn't persuasive enough". | **Biologic product**<br>Amyloid beta protein inhibitor which attacks amyloid plaques that are thought to cause symptoms of AD.<br><br>Product is the first in 17 years to be reviewed by FDA for treatment of AD. If approved, aducanumab would be the first treatment designed to delay progression of the disease. | **Therapy alternatives**<br>> Galantamine<br>> Rivastigmine |
| 03/17/2021 | **Brand Name TBD**<br>arimoclomol<br><br>Orphazyme | **Genetic disorder:**<br>Niemann-Pick disease type C | Oral<br><br>Niemann-Pick disease is a rare, life-threatening, genetic disorder that causes numerous problems including nerve damage.<br><br>Niemann-Pick disease types C1 and C2 are estimated to affect 1 in 150,000 individuals.<br><br>**FDA Designations:** Orphan Drug | Product is a "Molecular chaperone modulator" which increases production of certain proteins that help reduce tissue damage in nervous system diseases.<br><br>Current treatments for Niemann-Pick disease are symptomatic only. | **Therapy alternatives**<br>None |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 03/17/2021 | **Brand Name TBD**<br>idecabtagene vicleucel (aka "ide-cel", bb2121)<br><br>**Bluebird bio; Celgene; Bristol Myers Squibb** | **Blood cancer:**<br>Multiple myeloma, relapsed or refractory | IV<br><br>Given as a single lifetime dose.<br><br>Second most common form of blood cancer in the US, it is currently treatable to a certain extent but not curable. Median age at diagnosis is 69 years.<br><br>In 2020:<br>> About 32,270 new cases will be diagnosed<br>> About 12,830 deaths are expected to occur<br><br>**FDA designations:** Priority Review, Breakthrough Therapy | **Biologic product**<br>Chimeric antigen receptor T-cell (CAR-T) immunotherapy --To prepare ide-cel, a patient's own immune cells (T cells) are collected and genetically modified in a laboratory. Once infused back into the patient they find cells that express the antigen BCMA (found on myeloma cells) and activate a chain of events that will kill the tumor cells. | **Therapy alternatives**<br>> Triple combinations that include Darzalex (daratumumab) with dexamethasone and one of the following:<br>> Velcade (bortezomib) OR<br>> Kyprolis (carfilzomib) OR<br>> lenalidamide<br>> Blenrep |
| 03/31/2021 | **Fotivda**<br>tivozanib<br><br>**Aveo Ophthotech Kyowa Kirin** | **Kidney cancer:**<br>relapsed or refractory renal cell carcinoma as third- and fourth-line treatment | Oral<br><br>In 2020, about 28,000 new cases of kidney cancer will be diagnosed in women, and about 74,000 cases in men. Kidney cancer is more common in African Americans and American Indian /Alaska Natives, and the average age at diagnosis is 64.<br><br>Renal cell carcinoma (RCC) comprises about 9 out of 10 cases of kidney cancer. | Vascular endothelial growth factor receptor (VEGFR) tyrosine kinase inhibitor<br><br>FDA declined to approve tivozanib in 2013 and when Aveo filed the new application in March 2020, the company agreed to withdraw the NDA if study results did not demonstrate an increased chance of survival compared to sorafenib. Sorafenib, however, is not one of NCCN's preferred regimens for treating kidney cancer that has relapsed. | **Therapy alternatives**<br>> Cabozanitib<br>> Nivolumab |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 03/2021 | **Brand Name TBD**<br>fosdenopterin<br><br>**Alexion Pharmaceuticals**<br>**BridgeBio Origin Biosciences** | **Genetic disorder:**<br>Molybdenum cofactor deficiency | IV<br><br>Molybdenum cofactor deficiency causes intractable seizures and other nervous system issues in newborns, resulting in extremely poor prognosis.<br><br>Very rare condition, with estimated frequency of 1 in 100,000 to 200,000 newborns worldwide.<br><br>**FDA designations:** Breakthrough Therapy, Rare Pediatric Disease, Orphan Drug | Enzyme cofactor replacement therapy, directly replacing the deficient cofactor.<br><br>This is the first treatment to address the underlying cause of this condition. | **Therapy alternatives**<br>None |
| 1Q 2021 | **Brand name TBD**<br>ponesimod<br>(aka ACT-128800)<br><br>**Actelion JNJ Janssen** | **Multiple Sclerosis:**<br>Relapsing multiple sclerosis (MS) | Oral<br><br>Estimates of the prevalence of MS were updated in 2019 to reflect a figure twice as high as before, now indicating that more than 900,000 people in the US are living with MS. | Sphingosine 1-phosphate (S1P) receptor modulators<br><br>The ponesimod molecule is more likely than other drugs in its class (such as Gilenya and Mayzent) to bind to the cells where they exert their effects. It was shown superior to Aubagio in reducing MS flares in a head-to-head study. | **Therapy alternatives**<br>> Gilenya<br>> Mayzent<br>> Zeposia |
| 05/14/2021 | **Brand Name TBD**<br>pegcetacoplan<br><br>**Apellis** | **Blood disease:**<br>paroxysmal nocturnal hemoglobinuria (PNH) | Subcutaneous (may be self-administered)<br><br>PNH occurs when red blood cells with defective outer membranes are attacked by the body's immune system, causing anemia and other symptoms.<br>The estimated incidence of PNH is 1–10 cases per million people. The median age of onset is in the thirties, and it lasts lifelong.<br><br>**FDA Designation:** Priority Review | Targeted C3 therapy: reduces activation of the body's "complement cascade", which triggers the immune response causing damage in PNH. | **Therapy alternatives**<br>Soliris (eculizumab) |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 05/18/2021 | **Brand Name TBD**<br>avalglucosidase alfa (aka neoGAA)<br><br>**Sanofi** | **Genetic disorder:**<br>Pompe disease | IV<br><br>Pompe disease is a rare, genetic disease caused by an enzyme deficiency which results in damage to the liver and heart that is often life threatening.<br>Incidence is approximately 1 in 40,000 births in the United States.<br><br>**FDA Designations:** Orphan Drug | **Biologic product**<br>Lysosomal glycogen-specific enzyme, which helps the body to clear the substance that causes organ damage in this disease. | **Therapy alternatives**<br>Lumizyme (alglucosidase alfa) |
| 05/21/2021 | **Lonca**<br>loncastuximab tesirine<br><br>**ADC Therapeutics** | **Lymphoma (cancer):**<br>Diffuse large B cell lymphoma (DLBCL), relapsed or refractory | IV<br><br>DLBCL is the most common type of non-Hodgkins lymphoma. It was estimated that there would be approximately 25,000 new cases of DLBCL diagnosed in 2019 in the United States. | **Biologic product**<br>Cytotoxic agent anti-CD19 antibody; targets and kills DLBCL cells with the substance known as CD19 on their surface | **Therapy alternatives**<br>> Yescarta (axicabtagene ciloleucel)<br>> Kymriah (tisagenlecleucel) |
| 05/30/2021 | **Brand Name TBD**<br>belumosudil<br><br>**Kadmon** | **Bone marrow/stem cell transplant side effect:**<br>Chronic graft versus host disease (cGVHD) | Oral<br><br>GVHD occurs when stem cells given to a patient (as a treatment) attack the patient's own cells, causing damage to numerous organs and tissues. In severe cases, cGVHD can be fatal.<br><br>Approximately 14,000 patients in the United States are currently living with GVHD.<br><br>**FDA Designations:** Real-Time Oncology Review pilot program; Breakthrough Therapy Designation; Orphan Drug; Priority Review | Rho kinase (ROCK) inhibitor: blocks a process that contributes to inflammation and fibrosis involved in cGVHD. | **Therapy alternatives**<br>Imbruvica (ibrutinib) |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 06/05/2021 | **Ryplazim**<br>plasminogen<br><br>**ProMetic; Liminal BioSciences** | **Genetic disorder**:<br>congenital plasminogen deficiency | IV<br><br>Congenital plasminogen deficiency causes a build-up of substances on the eye and other mucous membranes, resulting in inflamed growths.<br><br>It is an ultra-rare genetic disorder affecting 1.6 per 1,000,000 people worldwide.<br><br>**FDA Designations**: Orphan Drug | **Biologic product**<br>Enzyme replacement therapy: directly replaces plasminogen, which is deficient in this condition. | **Therapy alternatives**<br>None |
| 06/25/2021 | **Brand Name TBD**<br>lonapegsomatropin (aka TransCon hGH)<br><br>**Ascendis** | **Growth hormone deficiency**:<br>pediatric and adult | Subcutaneous (may be self-administered)<br><br>Growth hormone deficiency not only causes stunted growth but may also result in reduced muscle and bone density and other effects for afflicted adults.<br><br>Adult GH deficiency has been estimated to affect 2 in 100,000 people annually (including those whose disease began in childhood).<br><br>The incidence of growth hormone deficiency in children is less than one in 3,000-10,000.<br><br>**FDA Designations**: Orphan Drug | **Biologic product**<br>Growth hormone<br><br>Similar in effectiveness with currently available growth hormone products, but only the second that is dosed on a weekly basis. | **Therapy alternatives**<br>Sogroyo (somapacitan-beco) |
| 06/2021 | **Brand name TBD**<br>infigratinib<br><br>**QED Therapeutics BridgeBio** | **Bile duct cancer**:<br>cholangiocarcinoma with FGFR2 gene abnormalities | Oral<br><br>Cholangiocarcinoma affects approximately 20,000 people in the US and EU each year. About 15-20% have the gene abnormalities treated by infigratinib. The condition is serious and often fatal. 5 year survival is about 9%.<br><br>**FDA Designations**: Priority Review; Real-Time Oncology Review pilot | Fibroblast growth factor receptor (FGFR) inhibitor: Inhibits abnormal signaling through the FGFR that can lead to several types of cancer. | **Therapy alternatives**<br>Surgery followed by chemotherapy with gemcitabine plus fluorouracil |

## Anticipated FDA Approvals *(continued)*

| Expected FDA Review Date | Drug Name / Manufacturer | Indication | Disease or Administration Comments | Clinical Comments | Therapy Alternatives |
|---|---|---|---|---|---|
| 2Q2021 | **Brand Name TBD**<br>tanezumab<br><br>**Pfizer Eli Lilly** | **Arthritis**:<br>Osteoarthritis (OA) pain | Subcutaneous<br><br>OA is a leading cause of disability. Patients with knee or hip OA also have a higher than usual risk of death due to cardiovascular events. | **Biologic product**<br>Antibody that blocks a chemical, nerve growth factor, involved in transmitting osteoarthritis pain.<br><br>First of this type of treatment for OA. Due to risk/benefit profile, it is not expected to replace standard pain control measures.<br><br>Studies showed no risk of addiction, dependence, or misuse. | **Therapy alternatives**<br>Combination of non-pharmacological approaches and analgesics (acetaminophen non-steroidal anti-inflammatory drugs and opioids). |
| CRL* issued on 10/2/2020 | **Ryoncil**<br>remestemcel-L<br><br>**Mesoblast** | **Bone marrow/stem cell transplant side effect**: Graft versus host disease (GVHD) refractory to steroids<br><br>Also has clearance from FDA for an Investigational New Drug application allowing expanded access compassionate use (outside of clinical trials) for respiratory distress syndrome due to COVID-19. | IV<br><br>GVHD occurs when stem cells given to a patient (as a treatment) attack the patient's own cells, causing damage to numerous organs and tissues.<br><br>If treatment with steroids fails, mortality rate can be up to 90%.<br><br>**FDA Designation**: Fast-track<br><br>**CRL Issues**:<br>> FDA requesting at least one additional study in adults and/or children to provide further evidence of efficacy. | **Biologic product**<br>Stem cell therapy created by culturing human stem cells (from healthy volunteers) in a laboratory. When given to a patient with GVHD, they act to interrupt the GVHD process.<br><br>There are no safe and effective therapies for children with steroid-refractory GVHD. | **Therapy alternatives**<br>None |

*CRL (Complete Response Letter) is a communication to a drug's manufacturer from the FDA indicating that the application for the drug cannot be approved in its present form.

## References

The above information was assembled from government and clinical resources for knowledge purposes only. Information and drugs were selected by clinicians based on therapy and potential clinical impact without any manufacture affiliations or conflicts of interest. Approval status, dates, and WAC price are subject to variation. This document should not be exclusively used for decision-making purposes. WAC pricing data should be used for benchmarking purposes only. Prices listed above should not be used alone to set or adjudicate any prices for reimbursement or purchasing functions or considered to be an exact price for a single product and/or manufacturer.

1. Adams, B. (2019, November 4). FDA tells Aveo no, again, for tivozanib as biotech tries to find yet another path forward. Retrieved from fiercebiotech.com: https://www.fiercebiotech.com/biotech/fda-tells-aveo-no-again-for-tivozanib-as-biotech-tries-to-find-yet-another-path-forward
2. Adams, B. (2020, April 24). GSK posts middling data from PD-1 Tesaro buyout drug. Retrieved from fiercebiotech.com: https://www.fiercebiotech.com/biotech/gsk-posts-middling-data-from-pd-1-tesaro-buyout-drug
3. Adams, B. (2020, September 4). Janssen sends off NDA for new multiple sclerosis drug ponesimod. Retrieved from fiercebiotech.com: https://www.fiercebiotech.com/biotech/janssen-sends-off-nda-for-new-multiple-sclerosis-drug-ponesimod
4. AIM with Immunotherapy Foundation. (2020, September 4). MSI-H/dMMR Endometrial Cancer. Retrieved from aimwithimmunotherapy.org: aimwithimmunotherapy.org/wp-content/uploads/2018/10/MSI-H_dMMr-Edometrial-Cancer-Final-1.pdf
5. Alnylam Pharmaceuticals. (2020, June 8). Alnylam Reveals Solid Lumasiran Results; Analyst Says Dicerna Better Pick. Retrieved from finance.yahoo.com: https://finance.yahoo.com/news/alnylam-reveals-solid-lumasiran-results-072429099.html
6. American Cancer Society. (2016, May 11). Why Are Stem Cell Transplants Used as Cancer Treatment? Retrieved from www.cancer.org: https://www.cancer.org/treatment/treatments-and-side-effects/treatment-types/stem-cell-transplant/why-stem-cell-transplants-are-used.html
7. American Cancer Society. (2020, September 1). Key Statistics About Multiple Myeloma. Retrieved from cancer.org: https://www.cancer.org/cancer/multiple-myeloma/about/key-statistics.html
8. American Cancer Society. (2020, June 14 ). Key Statistics for Lung Cancer. Retrieved from Cancer.org: https://www.cancer.org/cancer/lung-cancer/about/key-statistics.html
9. American Cancer Society. (2020, August 28). Key Statistics for Non-Hodgkin Lymphoma. Retrieved from cancer.org: https://www.cancer.org/cancer/non-hodgkin-lymphoma/about/key-statistics.html#:~:text=Non%2DHodgkin%20lymphoma%20(NHL),will%20be%20diagnosed%20with%20NHL.
10. ASH. (2020, May 1). Ropeginterferon Alfa-2b Leads to More Durable Responses Than Hydroxyurea in PV. Retrieved from ashclinicalnews.org: https://www.ashclinicalnews.org/news/literature-scan/ropeginterferon-alfa-2b-leads-durable-responses-hydroxyurea-pv/
11. AstraZeneca. (2018, February 15). Selumetinib granted Orphan Drug Designation by the US FDA for neurofibromatosis type 1. Retrieved from astrazeneca.com: https://www.astrazeneca.com/media-centre/press-releases/2018/selumetinib-granted-orphan-drug-designation-by-the-us-fda-for-neurofibromatosis-type-1-15022018.html#
12. B, A. (2019, August 16). FDA approves Celgene's bone marrow cancer treatment. Retrieved from Reuters: https://www.reuters.com/article/us-celgene-fda/fda-approves-celgenes-bone-marrow-cancer-treatment-idUSKCN1V61BS
13. Belluck, P. (2020, November 6). F.D.A. Panel Declines to Endorse Controversial Alzheimer's Drug. Retrieved from nytimes.com: https://www.nytimes.com/2020/11/06/health/aducanumab-alzheimers-drug-fda-panel.html
14. Berenbaum, F., Blanco, F., Guermazi, A., & et.al. (2020). Subcutaneous tanezumab for osteoarthritis of the hip or knee: efficacy and safety results from a 24-week randomised phase III study with a 24-week follow-up period. Ann Rheum Dis, 800-810.
15. BioCryst Pharmaceuticals. (2020, June 9). BioCryst Announces Berotralstat Expanded Access Program for Patients with Hereditary Angioedema in United States. Retrieved from finance.yahoo.com: https://finance.yahoo.com/news/biocryst-announces-berotralstat-expanded-access-201510895.html
16. Broderick, J. M. (2020, September 4). FDA Accepts Application for Ropeginterferon Alfa-2b for Polycythemia Vera. Retrieved from onclive.com: https://www.onclive.com/view/fda-accepts-application-for-ropeginterferon-alfa2b-for-polythycemia-vera
17. Brooks, M. (2020, November 24). FDA Clears First Drug to Treat Ultra-Rare Metabolic Disorder. Retrieved from Medscape.com: https://www.medscape.com/viewarticle/941520?src=
18. Businesswire. (2020, March 21). AVEO Oncology Announces Submission of New Drug Application to U.S. FDA for Tivozanib in Patients with Relapsed or Refractory Renal Cell Carcinoma. Retrieved from businesswire.com: https://www.businesswire.com/news/home/20200331005770/en/AVEO-Oncology-Announces-Submission-New-Drug-Application
19. Chander, V. (2020, August 5). U.S. FDA approves GlaxoSmithKline's blood cancer drug. Retrieved from reuters.com: https://www.reuters.com/article/us-gsk-multiplemyeloma-fda/u-s-fda-approves-glaxosmithklines-blood-cancer-drug-idUSKCN252058
20. Drugs.com. (2020, August 28). Aducanumab Approval Status. Retrieved from Drugs.com: https://www.drugs.com/history/aducanumab.html
21. Eiger Biopharmaceuticals. (2020, May 19). Eiger BioPharmaceuticals Announces FDA Acceptance of NDA for Filing with Priority Review of Zokinvy™ (lonafarnib) for Treatment of Progeria and Progeroid Laminopathies. Retrieved from prnewswire.com: https://www.prnewswire.com/news-releases/eiger-biopharmaceuticals-announces-fda-acceptance-of-nda-for-filing-with-priority-review-of-zokinvy-lonafarnib-for-treatment-of-progeria-and-progeroid-laminopathies-301062068.html
22. Eledrisi, M. S. (2018, June 26). Growth Hormone Deficiency in Adults. Retrieved from emedicine.medscape.com: https://emedicine.medscape.com/article/120767-overview#a6
23. EMD Serono. (2020, August 25). FDA Accepts Filing of New Drug Application for Tepotinib for the Treatment of Patients with Metastatic NSCLC with METex14 Skipping Alterations. Retrieved from Drugs.com: https://www.drugs.com/nda/tepotinib_200825.html
24. Express Scripts. (2020, November 30). November 30, 2020: Express Scripts Clinical News and Notes. St. Louis, MO, USA.
25. Fidler, B. (2020, August 13). FDA gives speedy approval to another Duchenne drug. Retrieved from Biopharmadive.com: https://www.biopharmadive.com/news/viltolarsen-duchenne-fda-approval-ns-pharma/583410/
26. G1 Therapeutics. (2020, August 28). Trilaciclib. Retrieved from g1therapeutics.com: https://www.g1therapeutics.com/pipeline/trilaciclib/
27. Gardner, J. (2020, August 25). Sarepta, after two dramatic approvals, gets an FDA review for next Duchenne drug. Retrieved from biopharmadive.com: https://www.biopharmadive.com/news/sarepta-after-two-dramatic-approvals-gets-an-fda-review-for-next-duchenne/584094/
28. Genetics Home Reference. (2020, June 9). Hereditary angioedema. Retrieved from ghr.nih.gov: https://ghr.nlm.nih.gov/condition/hereditary-angioedema#statistics
29. Globe Newswire. (2020, August 13). TG Therapeutics Announces FDA Acceptance of New Drug Application for Umbralisib as a Treatment for Patients with Previously Treated Marginal Zone Lymphoma and Follicular Lymphoma. Retrieved from marketwatch.com: https://www.marketwatch.com/press-release/tg-therapeutics-announces-fda-acceptance-of-new-drug-application-for-umbralisib-as-a-treatment-for-patients-with-previously-treated-marginal-zone-lymphoma-and-follicular-lymphoma-2020-08-13
30. Hart, L. L. ( 2019). Effect of trilaciclib, a CDK 4/6 inhibitor, on myelosuppression in patients with previously treated extensive-stage small cell lung cancer receiving topotecan. Journal of Clinical ONcology, Vol 37, Issue 15 suppl.
31. IBM Watson Health. (2020, August 28). Drugs that treat Alzheimer's disease. Retrieved from IBM Micromedex DRUGDEX (electronic version), Greenwood Village, CO: https://www.micromedexsolutions.com
32. IPD Analytics. (2019, May 22). CNS: Duchenne Muscular Dystrophy. Retrieved from IPD Analytics: https://secure.ipdanalytics.com/User/Pharma/RxStrategy/Page/eca66b03-236e-4d93-9e3a-88634b4a3bbb#section-group-26160
33. IPD Analytics. (2019). CNS: Duchenne Muscular Dystrophy. Retrieved from IPDanalytics.com: secure.ipdanalytics.com/User/Pharma/RxStrategy/Page/eca66b03-236e-4d93-9e3a-88634b4a3bbb#comment-groups
34. IPD Analytics. (2020 , September 11). IPD Analytics Market Forecast: Rhythm's Setmelanotide Expected to Expand Potential Patient Population After Launch . Bay Harbor Islands, FL, USA.
35. IPD Analytics. (2020). DRUG PIPELINE & TREND IMPACT. Bay Harbor Islands, FL: IPD Analytics.
36. IPD Analytics. (2020). Drug Pipeline Report. Bay Harbor Islands, FL: IPD Analytics.
37. IPD Analytics. (2020). Drug Pipeline Report for 2H 2020. Bay Harbor Islands, FL: IPD Analytics.
38. IPD Analytics. (2020, December 16). Endocrine and Metabolic Agents: Growth Hormone Deficiency. Retrieved from ipdanalytics.com: https://secure.ipdanalytics.com/User/Pharma/RxStrategy/Page/366790d0-f875-4633-954f-151ea398625c
39. IPD Analytics. (2020, December 16). Endocrine and Metabolic Agents: Pompe Disease. Retrieved from ipdanalytics.com: secure.ipdanalytics.com/User/Pharma/RxStrategy/Page/390257c1-0205-4ea0-ad0f-f96b1d1551f1#comment-groups
40. IPD Analytics. (2020, December 16). Hematologic: Paroxysmal Nocturnal Hemoglobinuria (PNH) Therapy. Retrieved from ipdanalytics.com: https://secure.ipdanalytics.com/User/Pharma/RxStrategy/Page/fce5a2bf-4822-43e7-8b95-5b88034a4454
41. IPD Analytics. (2020, October 8). IPD Analytics Market Forecast Pipeline Update: FDA Issues Complete Response Letter for Mesoblast's Ryoncil. Bay Harbor Islands, FL, USA.
42. IPD Analytics. (2020, December 10). IPD Analytics Market Forecast: Outlook for Amondys 45 . Bay Harbor Islands, FL, USA.
43. IPD Analytics. (2020, October 23). IPD Analytics Market Forecast: Outlook for Tanezumab. Bay Harbor Islands, FL, USA.
44. IPD Analytics. (2020, 4 13). IPD Analytics Pharmacy & Therapeutics Watch List. (email). Bay Harbor Islands FL.
45. IPD Analytics. (2020, March 9). IPD Analytics Pharmacy & Therapeutics Watch List. Bay Harbor Islands, FL.
46. IPD Analytics. (2020, February 24). IPD Analytics Pharmacy & Therapeutics Watch List. Bay Harbor Islands, FL, USA.
47. IPD Analytics. (2020, June 1). IPD Analytics Pharmacy & Therapeutics Watch List. Bay Harbor Islands, FL, USA.
48. IPD Analytics. (2020, August 24). IPD Analytics Pharmacy & Therapeutics Watch List. Bay Harbor Islands, FL, USA.
49. IPD Analytics. (2020, September 8). IPD Analytics Pharmacy & Therapeutics Watch List 9.8.20. Bay Harbor Islands, FL, USA .
50. IPD Analytics. (2020). Key Considerations in Managing Chimeric Antigen Receptor. Bay Harbor Islands, FL: IPD Analytics.
51. IPD Analytics. (2020). New Drug Review: Danyelza (naxitamab-gqgk). Bay Harbor Islands, FL: IPD Analytics.
52. IPD Analytics. (2020). New Drug Review: Zolkinvy (lonafarnib). Bay Harbor Islands, FL: IPD Analytics.
53. IPD Analytics. (2020). New Drug Review: Imcivree (setmelanotide). Bay Harbor Islands, FL: IPD Analytics.
54. IPD Analytics. (2020). New Drug Review: Orladeyo (berotralstat). Bay Harbor Islands, FL: IPD Analytics.
55. IPD Analytics. (2020). New Drug Review: Oxlumo (lumasiran). Bay Harbor Islands, FL: IPD Analytics.

## References *(continued)*


56. IPD Analytics. (2020, December 16). Oncology: Diffuse Large B-cell Lymphoma. Retrieved from ipdanalytics.com: https://secure.ipdanalytics.com/User/Pharma/RxStrategy/Page/22873d30-b997-436c-80f3-e2b653d25317
57. IPD Analytics. (2020). Podcast Transcript: CAR-T Approval and Pipeline Review. Bay Harbor Islands, FL: IPD Analytics.
58. IPD Analytics. (2020). Potential High-Cost Drug Approvals Expected Through 2021. Bay Harbor Islands, FL: IPD Analytics.
59. Kadmon Holdings, Inc. (2020, November 30). Kadmon Announces Submission of New Drug Application to the U.S. FDA for Belumosudil in Patients with Chronic Graft-Versus-Host Disease. Retrieved from Ddrugs.com: https://www.drugs.com/nda/belumosudil_200930.html
60. Kurtzburg, J., Abdel-Azim, H., Carpenter, P., & et.al. (2020). A Phase 3, Single-Arm, Prospective Study of Remestemcel-L, Ex Vivo Culture-Expanded Adult Human Mesenchymal Stromal Cells for the Treatment of Pediatric Patients Who Failed to Respond to Steroid Treatment for Acute Graft-versus-Host Disease. Biol Blood Marrow Transplant, 845-854.
61. Lymphoma Research Foundation. (2020, September 4). Marginal Zone Lymphoma. Retrieved from lymphoma.org: https://lymphoma.org/aboutlymphoma/nhl/mzl/
62. Mayo Clinic. (2020, September 4). Polycythemia vera. Retrieved from mayoclinic.org: https://www.mayoclinic.org/diseases-conditions/polycythemia-vera/symptoms-causes/syc-20355850
63. MedlinePlus. (2020, December 16). Niemann-Pick disease. Retrieved from medlineplus.gov: https://medlineplus.gov/genetics/condition/niemann-pick-disease/#frequency
64. Memorial Sloan Kettering. (2020, June 12). Neuroblastoma. Retrieved from mskcc.org: https://www.mskcc.org/pediatrics/cancer-care/types/neuroblastoma?msclkid=17c6e9e402e317b1a760b7003c9fce33&utm_source=bing&utm_medium=cpc&utm_campaign=MSK%20Kids_US&utm_term=how%20common%20neuroblastoma&utm_content=Neuroblastoma_Information
65. Mesoblast Ltd. (2019, May 29). Mesoblast Initiates Rolling Submission of Biologics License Application (BLA) to U.S. FDA for remestemcel-L in the Treatment of. Retrieved from bloomberg.com: https://www.bloomberg.com/press-releases/2019-05-30/mesoblast-initiates-rolling-submission-of-biologics-license-application-bla-to-u-s-fda-for-remestemcel-l-in-the-treatment-of
66. Mesoblast Ltd. (2020, April 6). FDA Clears IND for Remestemcel Use in COVID-19 ARDS Patients. Retrieved from drugs.com: https://www.drugs.com/clinical_trials/fda-clears-ind-remestemcel-covid-19-ards-patients-18518.html
67. Nadeem, D. (2020, August 7). Alzheimer's drug from Biogen to get speedy U.S. review. Retrieved from Reuters.com: https://www.reuters.com/article/us-biogen-fda-aducanumab/alzheimers-drug-from-biogen-to-get-speedy-u-s-review-idUSKCN2531OC
68. National Cancer Institute. (2020, December 2). Neuroblastoma Treatment (PDQ®)–Health Professional Version. Retrieved December 14, 2020, from Cancer.gov: https://www.cancer.gov/types/neuroblastoma/hp/neuroblastoma-treatment-pdq#_138_toc
69. National MS Society. (2020, September 4). Landmark Study Estimates Nearly 1 Million in the U.S. Have Multiple Sclerosis. Retrieved from nationalmssociety.org: https://www.nationalmssociety.org/About-the-Society/News/Landmark-Study-Estimates-Nearly%C2%A01-Million-in-the-U
70. NCCN. (2020). NCCN Clinical Practice Guidelines in Oncology: B-cell Lymphomas v4.2020. Philadelphia: NCCN.
71. NCCN. (2020). NCCN Clinical Practice Guidelines in Oncology: Hepatobiliary Cancers. Philadelphia: NCCN.
72. NCCN. (2020). NCCN Clinical Practice Guidelines in Oncology: Kidney Cancer v 1.2021. Philadelphia: NCCN.
73. NCCN. (2020). NCCN Clinical Practice Guidelines in Oncology: Multiple Myeloma v1.2021. Philadelphia: NCCN.
74. NCCN. (2020). NCCN Clinical Practice Guidelines in Oncology: Non-Small Cell Lung Cancer V32020. Philadelphia: NCCN.
75. NCCN. (2020). NCCN Clinical Practice Guidelines in Oncology: Small Cell Lung Cancer V3.2020. Philadelphia: National Comprehensive Cancer Network.
76. NCI. (2020, September 4). Mismatch repair deficiency. Retrieved from Cancer.gov: https://www.cancer.gov/publications/dictionaries/cancer-terms/def/mismatch-repair-deficiency
77. NORD. (2020, December 16). Pompe Disease. Retrieved from rarediseases.org: https://rarediseases.org/rare-diseases/pompe-disease/
78. Pediatric Endocrine Society. (2020, June 17). Growth Hormone Deficiency:. Retrieved from pedsendo.org: https://pedsendo.org/patient-resource/growth-hormone-deficiency/
79. Porter, D. L., & Maloney, D. G. (2020, August 28). Cytokine release syndrome (CRS). Retrieved from UpToDate, Waltham MA: https://www.uptodate.com/contents/cytokine-release-syndrome-crs
80. Reuters. (2020, June 2). BRIEF-Y-Mabs Announces U.S. FDA Acceptance Of Biologics License Application For Danyelza(Naxitamab). Retrieved from Reuters.com: https://www.reuters.com/article/brief-y-mabs-announces-us-fda-acceptance-idUSASA00SFW
81. Silva, J. C. (2020, April 16). Idecaptagene Cicleucel (Ide-Cel; Previously bb2121). Retrieved from Immuno-oncologynews.com: https://immuno-oncologynews.com/bb2121/
82. Singh, M. (2020, December 1 ). FDA accepts BridgeBio Pharma/QED's infigratinib application for bile duct cancer. Retrieved from seekingalpha.com: https://seekingalpha.com/news/3640300-fda-accepts-bridgebio-pharma-qeds-infigratinib-application-for-bile-duct-cancer
83. Slater, H. (2020, July 24). FDA Approves Brexucabtagene Autoleucel for Relapsed/Refractory Mantle Cell Lymphoma. Retrieved from cancernetwork.com: https://www.cancernetwork.com/view/fda-approves-brexucabtagene-autoleucel-for-relapsed-refractory-mantle-cell-lymphoma
84. Slater, H. (2020, September 22). FDA Grants Priority Review to Idecabtagene Vicleucel for Multiple Myeloma. Retrieved from cancernetwork.com: https://www.cancernetwork.com/view/fda-grants-priority-review-to-idecabtagene-vicleucel-for-multiple-myeloma
85. Taiho Oncology. (2020, September 1). Taiho Oncology Patient Support. Retrieved from taihopatientsupport.com: https://www.taihopatientsupport.com/
86. Tefferi, A. (2020, September 4). Polycythemia Vera. Retrieved from rarediseases.org: https://rarediseases.org/rare-diseases/polycythemia-vera/
87. UpToDate. (2020, June 8). Selumetinib: Drug information. Retrieved from UpToDate.com: https://www.uptodate.com/contents/selumetinib-drug-information?search=koselugo&source=panel_search_result&selectedTitle=1~11&usage_type=panel&kp_tab=drug_general&display_rank=1#F54317217
88. vandeDonk, N. W. (2018, January 4). CD38 antibodies in multiple myeloma: back to the future. Retrieved from ashpublications.org: https://ashpublications.org/blood/article/131/1/13/107710/CD38-antibodies-in-multiple-myeloma-back-to-the
89. Weintraub, A. (2019, November 14). AstraZeneca's selumetinib moves past cancer failures to nab FDA priority review in neurofibromatosis. Retrieved from fiercepharma.com: https://www.fiercepharma.com/pharma/astrazeneca-s-selumetinib-moves-past-cancer-failures-to-nab-fda-priority-review-rare
90. William Lumry, M. M. (2020, March 13). Relative Reductions in Attack Rate With Prophylactic Berotralstat (BCX7353) in Patients With Hereditary Angioedema: Responder Analysis From the APeX-2 Study. Retrieved from biocryst.com: https://www.biocryst.com/wp-content/uploads/2020/03/AAAAI_Responder-Analysis_Poster_FINAL.pdf
91. Y-mAbs Therapeutics. (2020, June 12). Naxitamab for High-Risk Neuroblastoma Patients With Primary Refractory Disease or Incomplete Response to Salvage Treatment in Bone and/or Bone Marrow. Retrieved from ClinicalTrials.gov: https://clinicaltrials.gov/ct2/show/NCT03363373#armgroup
92. Y-mAbs Therapeutics,Inc. (2020, June 12). About the Y-mAbs development pipeline of therapies. Retrieved from ymabs.com: https://ymabs.com/research-development/pipeline/