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2ND QUARTER 2021 PIPELINE REPORT:
MEDICAL AND PHARMACY BENEFIT DRUGS

This Pipeline Report is focused on potentially budget-busting medications. We bring you information on

- What these drugs are used for;
- How common those conditions are;
- Current treatments for those conditions;
- How much the current treatments cost; and
- What to expect when these drugs have been approved.

Most importantly, as a trusted advisor, Confidio recommends viable strategies for managing these expensive treatments.

There are three sections in this report:

- **Top Five:** our pick of products recently approved or pending approval that we believe warrant the most attention
- **Recent FDA Approvals:** detailed information on recently approved high-cost drugs under both the medical and pharmacy benefit
- **Anticipated Approvals:** summary table for potentially high-cost products under development

TOP FIVE:

Recently approved or pending approval products that may have a significant impact on drug costs in the medical or pharmacy benefit.

WHAT *Drug & condition*	**WHEN** *FDA approval date*	**WHERE** *Probable benefit coverage*	**WHY** *What earned this drug a Top 5 placement*	**ACTIONS** *Strategies for managing cost*
Amondys 45 (casimersen) For one subset of Duchenne muscular dystrophy	Approved 2/25/2021	Medical benefit	• Was approved based on surrogate markers; effect on disease state symptoms and progression is still being studied • Cost could be up to $1 million per year • Low condition prevalence* at 1.6 cases per 100,000 population	• Home infusion; apply inventory management • Prior authorization or exclude as not medically necessary (until positive clinical outcomes demonstrated) • Manufacturer copay assistance program • Stop-loss • Support groups and complex case management
Cosela (trilaciclib) To prevent chemo-induced immune suppression	Approved 2/12/2021	Medical benefit	• New mechanism of action which hopefully will improve outcomes • Can be used to prevent immune suppression rather than react to it, as current therapy does	• Prior authorization to prevent use when immune suppression is not likely • G1 Commercial Copay Program • Oncology case management
Brand name TBD (gold nanocrystal) For Lou Gehrig's disease (ALS)	Anticipated approval, March 2021	Pharmacy benefit	• New mechanism for treating this devastating disease	Pending approval
Brand name TBD (vosoritide) To treat one form of dwarfism	Anticipated approval, August 2021	Pharmacy benefit	• This is the first product available to treat the underlying disease (achondroplasia); current treatment manages symptoms but does not prevent/reverse dwarfism	Pending approval
Brand name TBD (aducanumab) To treat Alzheimer's disease	Anticipated approval, June 2021	Medical benefit	• Relatively common condition • First treatment available to modify the disease (current treatments delay onset/progression or treat symptoms only)	Pending approval

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RECENT FDA APPROVALS

Pharmacy Benefit

Fotivda (tivozanib): Aveo Ophthotech, Kyowa Kirin

- **Approval date: 3/10/2021**
- **Pharmacy benefit**
  - The product is self-administered as an oral capsule, without regard to food intake.
- **Indication and frequency**
  Recurrent kidney cancer (relapsed or refractory renal cell carcinoma as third - or fourth-line treatment); incidence* is about 3 cases per 10,000 population.
- **Cost factors**
  - Annual cost: $313,950
  - Therapeutic alternative annual treatment cost: Cabometyx (cabozanitib), $263,408; or Opdivo (nivolumab), $173,659
- **Therapeutic impact**
  - Incremental improvement
    - Approved based on one study comparing it to sorafenib, which is currently neither a preferred nor recommended regimen for this type and stage of cancer.
    - May have advantage over Opdivo (which is administered IV) and Cabometyx (which requires dosing on an empty stomach).
- **Management strategies**
  - Inquire into PBM programs
  - Prior authorization to ensure trial/failure of first-line therapies
  - Copay assistance may be available through AVEO ACE
  - Oncology case management

Ponvory (ponesimod): Janssen, J&J

- **Approval date: 3/19/2021**
- **Pharmacy benefit**
  - Once daily oral therapy
- **Indication and frequency**
  Multiple sclerosis, relapsing forms (MS); prevalence* of MS is 2 cases per 1000 population.
- **Cost factors**
  - Annual cost, unknown as of publication date; likely to be similar to Gilenya, Mayzent, Zeposia
  - Therapeutic alternative annual treatment cost: Gilenya (fingolimod), $110,000; Mayzent (siponimod), $98,000; or Zeposia (ozanimod), $90,000
- **Therapeutic impact**
  - Incremental improvement
    - Fourth drug in its class.
    - May bind more strongly to target cells than other drugs in its class, possibly improving effectiveness.
- **Management strategies**
  - Inquire into PBM programs
  - Depending on price, consider step therapy to require less expensive products in this class

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RECENT FDA APPROVALS *(continued)*

Pharmacy Benefit

Tepmetko (tepotinib): Merck, KGaA, EMD Serono

- **Approval date: 2/3/2021**
- **Pharmacy benefit**
  - Once daily oral therapy
- **Indication and frequency**
  Lung cancer (metastatic non-small cell lung cancer [NSCLC], in tumors with MET gene alterations); incidence* of NSCLC with MET gene alternations is between 1.7 and 2.8 cases per 10,000 population.
- **Cost factors**
  - Annual cost, $250,788
  - Therapeutic alternative annual treatment cost: Tabrecta (capmatinib), $246,861
- **Therapeutic impact**
  - FDA Designation: Priority Review, Accelerated Approval, Real-Time Oncology Review pilot, Breakthrough Therapy, Orphan Drug
  - Incremental improvement
    - This is the second treatment approved for this specific patient population.
    - Was not tested in comparison to Tabrecta, but in separate trials, effectiveness appears similar.
- **Management strategies**
  - Inquire into PBM programs
  - Prior authorization to ensure MET exon 14 skipping alterations
  - Copay assistance may be available through EMD Serono Oncology Navigation Center™ (ONC)
  - Oncology case management

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RECENT FDA APPROVALS *(continued)*

Pharmacy Benefit

Ukoniq (umbralisib tosylate): TG Therapeutics

- **Approval date: 2/5/2021**
- **Pharmacy benefit**
  - Once daily oral therapy
- **Indication and frequency**
  Blood cancers (marginal zone lymphoma [MZL] and follicular lymphoma [FL]), after trial of other treatment regimens; incidence* of MZL, 2 cases per 100,000 population; of FL, 5 cases per 100,000 population.
- **Cost factors**
  - Annual cost, $190,800
  - Therapeutic alternative annual treatment cost, combination regimens such as "R-CHOP" $47,000 OR bendamustine + obinutuzumab $113,000
- **Therapeutic impact**
  - Incremental improvement
    - There are numerous alternatives for these conditions, and Ukoniq has not been tested against any of them.
    - Theoretical advantages include a second mechanism of action and possibly less severe side effects.
    - FDA designations: Breakthrough Therapy, Priority Review, Accelerated Approval
- **Management strategies**
  - Inquire into PBM programs
  - Prior authorization/step therapy
  - Copay assistance may be available through TG Patient Support
  - Oncology case management

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Amondys 45 (casimersen): Sarepta Therapeutics

- **Approval date: 2/25/2021**
- **Medical benefit**
  - The product is given via intravenous infusion over 35-60 minutes once weekly.
  - Typically administered via home infusion, physician office or infusion center.
- **Indication and frequency**
  - One subset of Duchenne muscular dystrophy (DMD) (patients with a gene mutation amenable to exon 45 skipping); prevalence* is 1.6 cases per 100,000 population.
  - There are three other specialty drugs available for other forms of DMD.
- **Cost factors**
  - Annual cost: Up to $1,000,000 per year (weight-based dosing); priced at parity to other DMD specialty drugs.
  - Alternative therapy annual treatment cost: N/A--symptomatic only
- **Therapeutic impact**
  - Incremental improvement
    - Amondys 45 is the first product specific to DMD with exon 45 skipping. As such, it expands the number of patients for whom a key marker (dystrophin levels) of the disease may be improved. However, the degree of improvement for that marker, and impact it actually has on muscle deterioration and weakness, are still under study in ongoing clinical trials.
    - FDA designations: Orphan Drug, Fast Track, Priority Review; FDA granted an accelerated approval with ongoing clinical benefit confirmatory trials.
- **Management strategies**
  - Stop loss insurance
  - Prior authorization to require genetic testing
  - Copay assistance may be available through SareptAssist
  - Care setting: evaluate options for clinically appropriate, least costly site of care
  - Home infusion inventory management
  - Patient support groups
  - Complex case management

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Breyanzi (lisocabtagene maraleucel), Bristol Myers Squibb

- **Approval date: 2/5/2021**
- **Medical benefit**
  - Single IV dose therapy administered in a certified healthcare facility.
- **Indication and frequency**
  Relapsed or refractory blood cancer (large B-cell lymphoma (LBCL), after two or more lines of systemic therapy); incidence* is approximately 1-2 cases per 10,000 population.
- **Cost factors**
  - Annual cost: $410,300 (one-time dose)
  - Alternative therapy annual treatment cost: Yescarta (axicabtagene ciloleucel), or Kymriah (tisagenlecleucel), both priced at $373,000.
  - Note that Breyanzi, Yescarta and Kymriah are once-per-lifetime doses that are given following chemotherapy to deplete the body's immune cells. These prices do not reflect the cost of the chemotherapy.
- **Therapeutic impact**
  - Incremental improvement
    - Comparative trials have not been done; this is the third CAR-T cell therapy available for lymphoma (following Kymriah and Yescarta).
    - May have a more rapid and durable response rate.
    - May have a lower rate than Kymriah and Yescarta of a life threatening complication (cytokine release syndrome) associated with CAR-T cell therapies (all three have FDA "black boxes" for this).
- **Management strategies**
  - Stop loss insurance
  - Inquire into PBM programs
  - Prior authorization/step therapy
  - Copay assistance may be available through Cell Therapy 360
  - Complex case management
  - Center of Excellence / certified healthcare facilities for administration

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Cosela (trilaciclib): G1 Therapeutics, Boehringer Ingelheim

- **Approval date: 2/12/2021**
- **Medical benefit**
  - The product is given via intravenous infusion within 4 hours prior to chemotherapy each day chemotherapy; is administered.
  - Typically administered in an outpatient infusion center or oncology infusion center.
- **Indication and frequency**
  Prevention of chemotherapy-associated immune suppression (in patients with extensive stage small cell lung cancer being treated with chemotherapy): incidence*: approximately 6 cases in 100,000 population.
- **Cost factors**
  - Cost will depend on the chemotherapy regimen.
  - Cost range $34,000 to $56,000 over four 21-day chemotherapy cycles.
  - Alternative therapy annual treatment cost:
    - Granulocyte colony-stimulating factors (GCSFs) to treat low white blood count: filgrastim and pegfilgrastim. Cost of GCSFs can range from $60,000-100,000 per year.
    - Erythropoiesis-stimulating agents (ESAs) to treat anemia: epoetin and darbepoetin. Cost can range from $7,500-17,000 per year.
  - In clinical studies, approximately 35% of patients still required treatment with ESAs or GCSFs in addition to Cosela. In the same trials, 67% of patients on placebo required treatment with ESAs or GCSFs.
- **Therapeutic impact**
  - Incremental improvement
    - Is unique in its mechanism of action and in that it is given before toxicity begins.
    - FDA designations: Breakthrough Therapy, Priority Review
    - Use of Cosela reduced hospitalizations compared to placebo.
- **Management strategies**
  - Prior authorization to prevent inappropriate off-label use
  - Copay assistance may be available through G1 Commercial Copay Program
  - Oncology case management
  - Care setting: evaluate options for clinically appropriate, least costly site of care

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Evkeeza (evinacumab-dgnb): Regeneron

- **Approval date: 2/11/2021**
- **Medical benefit**
  - The product is administered once monthly via intravenous infusion; if deemed appropriate may be administered at home by the patient's caregiver.
- **Indication and frequency**
  Add-on therapy for the inherited form of high cholesterol (severe homozygous familial hypercholesterolemia); prevalence* is approximately 1 case in 250,000 population.
- **Cost factors**
  - Annual cost: $450,000-579,000 (varies based on patient weight) .
  - Previous annual treatment cost: Juxtapid (lomitapide), $583,000 or plasmapheresis (a blood "cleansing" procedure performed every 1-2 weeks), $88,000-225,000.
- **Therapeutic impact**
  - Incremental improvement
    - May be more palatable than plasmapheresis; may have less liver toxicity than Juxtapid.
    - Has not been studied in comparison to plasmapheresis or Juxtapid.
- **Management strategies**
  - Stop loss insurance
  - Prior authorization to require genetic test/step therapy
  - Copay assistance may be available through myRARE Copay Card
  - Care setting: After therapy initiation with first doses administered by a healthcare provider (most likely in a hospital setting); may transition to home infusion initially with nursing support and caregiver training.
  - Home infusion inventory management
  - Complex case management

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Nulibry (fosdenopterin): BridgeBio, Origin Biosciences

- **Approval date: 2/26/2021**
- **Medical benefit**
  - The product is administered once daily via intravenous infusion; may be administered at home by the patient's caregiver as appropriate.
- **Indication and frequency**
  - Molybdenum cofactor deficiency is a rare genetic disorder that causes intractable seizures and other nervous system issues in newborns due to accumulation of toxic sulfite metabolites.
  - The condition typically presents in the first few days of life with a median survival of 4 years.
  - < 1 case occurs per 100,000 live births; affects less than 150 patients globally.
- **Cost factors**
  - Annual cost: $852,717 (based on 18 kg/40 pound child)
  - Alternative therapy annual treatment cost: N/A—other therapies only treat symptoms and complications of the disease.
- **Therapeutic impact**
  - Major advance
    - FDA designations: Breakthrough Therapy, Priority Review, Orphan Drug
    - First product ever to treat this disease.
- **Management strategies**
  - Stop loss insurance
  - Prior authorization that requires genetic testing to confirm diagnosis
  - Monitor newborn medical claim outliers for early identification
  - Copay assistance may be available through ForgingBridges | NULIBRY Copay Assistance Program
  - Care setting: After therapy initiation with first doses administered by a healthcare provider (most likely in a hospital setting), ongoing daily administrations will most likely transition to home infusion initially with nursing support and caregiver training.
  - Home infusion inventory management
  - Complex case management support
  - Centers of excellence or rare condition specialist

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Pepaxto [fka Ygalo] (melphalan flufenamide [fka melflufen]): Oncopeptides

- **Approval date: 2/26/2021**
- **Medical benefit**
  - The product is given via intravenous infusion on the first day of each 28-day chemotherapy treatment cycle.
  - Typically administered in an outpatient infusion center or oncology infusion center.
- **Indication and frequency**
  Blood cancer (relapsed or refractory multiple myeloma); incidence* is approximately 1 case per 10,000 population.
- **Cost factors**
  - Annual cost: $205,833
  - Therapeutic alternative annual treatment cost:
    - Triple combinations that include Darzalex (daratumumab) with dexamethasone and Velcade (bortezomib) OR Kyprolis (carfilzomib) OR lenalidamide, range $169,207-$354,639
    - Blenrep (belantamab), $246,240
- **Therapeutic impact**
  - Incremental improvement
    - Other treatments available for this patient population, but none provide high response rates or improve survival to a large extent, so this provides another alternative.
    - Has not been compared head-to-head with current treatments; may or may not be superior.
    - FDA designation: Accelerated Approval.
- **Management strategies**
  - Step therapy: Limit to FDA labeled indication at this time, which specifies number and types of prior therapies that must have been tried first.
  - Copay assistance may be available through PEPAXTO® CoPay Card Program
  - Oncology case management
  - Care setting: evaluate options for clinically appropriate, least costly site of care.

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ANTICIPATED FDA APPROVALS

Pharmacy Benefit

Anticipated Approval Date	Brand Name	Generic Name	Anticipated Benefit Placement and Route of Administration	Indication/Use	Condition Incidence or Prevalence*	Therapeutic Impact	New Product Anticipated Annual Cost	Alternative Therapy on Market and Annual Cost
1/2021		Zilucoplan	Pharmacy--Subcutaneous	Myasthenia gravis (nervous system disorder causes weakness, vision changes, shortness of breath and other symptoms)	Incidence* < 1 case per 100,000 population	Other products with same mechanism on market	TBD	Soliris @ $700K
2/2021		Vutrisiran	Pharmacy--Subcutaneous	Polyneuropathy of hereditary transthyretin-mediated amyloidosis (nervous system dysfunction from buildup of abnormal proteins)	Prevalence* in US is < 6,400 patients		TBD	Onpattro @ $282K and Tegsedi @ $386K
3/2021		Arimoclomol	Pharmacy--Oral	Niemann-Pick disease type C (rare genetic life-threatening disorder that causes nerve damage)	Incidence* < 1 case per 100,000 live births		TBD	
3/2021		Gold nanocrystal	Pharmacy--Oral	Amyotrophic lateral sclerosis (ALS, Lou Gehrig's disease)	Incidence* 2 cases per 100,000 population	First product in class; not curative	TBD	Riluzole (generic) @ $22K or Radicava @ $150K
3/2021		Verdiperstat	Pharmacy--Oral	Multiple system atrophy (very rare nervous system disorder)	Incidence* < 1 case per 10,000 population	Not curative; may delay progression	TBD	

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ANTICIPATED FDA APPROVALS *(continued)*

Pharmacy Benefit

Anticipated Approval Date	Brand Name	Generic Name	Anticipated Benefit Placement and Route of Administration	Indication/Use	Condition Incidence or Prevalence*	Therapeutic Impact	New Product Anticipated Annual Cost	Alternative Therapy on Market and Annual Cost
1Q2021		Ropeginterferon alfa-2b	Pharmacy--Subcutaneous	Polycythaemia vera (blood cancer where overabundance of red blood cells causes serious blood 'thickening')	Prevalence* 2 cases per 100,000 population	Early clinical trials show ropeginterferon alfa-2b resulting in more complete remissions after 3 years compared to Hydrea	TBD	Hydrea @ $888
5/2021		Pegcetacoplan	Pharmacy--Subcutaneous	Paroxysmal nocturnal hemoglobinuria (PNH) (blood disease where immune cells attack red blood cells causing anemia)	Estimated 5,000-6,000 individuals in the US affected by PNH		TBD	Soliris @ $522K
5/2021		Belumosudil	Pharmacy--Oral	Chronic graft versus host disease (cGVHD) (bone marrow/stem cell transplant side effect that can be fatal)	Approximately 9,000 allogeneic transplants were performed in 2018; GVHD occurs in 30% to 60% of such transplants	If approved, will be first FDA-approved drug for cGVHD in patients 12 to 18 years of age	TBD	Imbruvica @ $180K
6/2021		Infigratinib	Pharmacy--Oral	Cholangiocarcinoma with FGFR2 gene abnormalities: (often-fatal bile duct cancer)	Incidence* < 1 case per 100,000 population	Only 9% of these patients survive 5 years	TBD	Surgery plus chemotherapy; range of costs
2Q2021		Tanezumab	Pharmacy--Subcutaneous	Osteoarthritis pain	Prevalence* 32.5 million US adults, approximately 10% of US population	First of this type of treatment for osteoarthritis; due to risk/benefit profile, not expected to replace standard pain control measures	TBD	Numerous over-the-counter non-prescription products

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ANTICIPATED FDA APPROVALS *(continued)*

Pharmacy Benefit

Anticipated Approval Date	Brand Name	Generic Name	Anticipated Benefit Placement and Route of Administration	Indication/Use	Condition Incidence or Prevalence*	Therapeutic Impact	New Product Anticipated Annual Cost	Alternative Therapy on Market and Annual Cost
7/2021		Avacopan	Pharmacy--Oral	ANCA associated vasculitis (rare disorder of blood vessels causing kidney and lung problems)	Incidence* < 1 case per 10,000 population	Unique mechanism of action	TBD	Rituxan @ $19K
7/2021		Bimekizumab	Pharmacy--Subcutaneous	Plaque psoriasis (common skin condition)	Incidence* 8 cases per 10,000 population	Multiple products on market with similar mechanism of action	TBD	Remicade @ $25K, Stelara @ $88K, Cosentyx @ $124K and others
8/2021		Sotorasib	Pharmacy--Oral	Non-small cell lung cancer with a specific genetic mutation	Incidence* 8 cases per 100,000 population	First product to target the genetic mutation	TBD	Keytruda + Cisplatin + Alimta @ $227K
8/2021		Vosoritide	Pharmacy--Subcutaneous	Achondroplasia, a rare, genetic type of dwarfism	Incidence* 3-5 cases per 100,000 live births	First product to treat condition	TBD	None
9/2021		Dersimelagon	Pharmacy--Oral	X-linked protoporphyria (extremely rare genetic condition that causes severe pain, burning and itching in sun-exposed skin)	Incidence* unknown		TBD	Scenesse @ $271K

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ANTICIPATED FDA APPROVALS *(continued)*

Medical Benefit

Anticipated Approval Date	Brand Name	Generic Name	Anticipated Benefit Placement	Indication/Use	Condition Incidence or Prevalence*	Comments	New Product Anticipated Annual Cost	Alternative Therapy on Market and Annual Cost
4Q2020		Dostarlimab	Medical--Intravenous	Specific types of uterine cancer	6 cases per 100,000 population	Will be 7th drug in this class	TBD	Keytruda @ $177K
1/2021		Nipocalimab	Medical--Intravenous	Hemolytic disease of the fetus and newborn (causes life-threatening anemia)	3-80 cases per 100,000 live births		TBD	None
3/2021		Idecabtagene vicleucel	Medical--Intravenous	Relapsed or refractory multiple myeloma (blood cancer)	Incidence* 1 case per 10,000 population	Given as a single lifetime dose	TBD	Multiple therapies ranging from $170K to $350K
5/2021		Avalglucosidase alfa	Medical--Intravenous	Pompe disease (rare genetic enzyme deficiency damages liver and heart)	Incidence* < 1 case per 10,000 live births		TBD	Lumizyme @ $600K
5/2021	Lonca	Loncastuximab tesirine	Medical--Intravenous	Lymphoma (blood cancer)	Incidence* 7.5 cases per 100,000 population		TBD	Yescarta and Kymriah each at $370K Or Breyanzi @ $400K
6/2021		Aducanumab	Medical--Intravenous	Alzheimer's disease	Incidence* 1.5 cases per 1,000 population	First disease modifying treatment	Estimates range from $20K to $100K	Razadyne @$2K or Namenda @$3K

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ANTICIPATED FDA APPROVALS *(continued)*

Medical Benefit

Anticipated Approval Date	Brand Name	Generic Name	Anticipated Benefit Placement	Indication/Use	Condition Incidence or Prevalence*	Comments	New Product Anticipated Annual Cost	Alternative Therapy on Market and Annual Cost
6/2021	Ryplazim	Plasminogen	Medical--Intravenous	Congenital plasminogen deficiency (enzyme deficiency that results in inflamed growths on mucous membranes and eyes)	Prevalence* is 1.6 cases per 1,000,000 population		TBD	
7/2021		Narsoplimab	Medical--Intravenous	Bone marrow/stem cell transplant-associated thrombotic microangiopathy (TM-TMA)(can cause anemia, bleeding, organ damage or death)	Approximately 9000 allogeneic transplants were performed in 2018. TA-TMA may occur in 3-5% of allogeneic transplants.		TBD	Defitelio @ $170K
8/2021		Vicineum	Medical--Administered into the bladder	Resistant bladder cancer	Incidence* 2 cases per 10,000 population		TBD	Gemcitabine @ $12K or Keytruda @ $150K
9/2021		Efgartigimod	Medical--Intravenous	Myasthenia gravis (nervous system disorder)	Incidence* < 1 per 100,000 lives		TBD	Soliris @ $700K
First half 2021	Leukotac	Inolimomab	Medical--Intravenous	Acute graft vs. host disease (GVHD) (allogeneic bone marrow/stem cell transplant side effect that can be fatal)	Approximately 9,000 allogeneic transplants were performed in 2018; GVHD occurs in 30-60% of transplants		TBD	Jakafi @ $170K

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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 manufacturer 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.

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