

3RD 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	HOW Strategies for managing cost
vosoritide For the treatment of achondroplasia, the most common type of dwarfism (genetic)	Anticipated approval, August 2021	Pharmacy benefit	<ul style="list-style-type: none"> While this product’s indication is extremely rare, it would revolutionize treatment of this condition, likely commanding a premium price. Orphan drug, Priority Review 	<ul style="list-style-type: none"> Prior authorization to ensure member has the indicated type of dwarfism Patient support groups Member inventory management (MIM) Education
Aduhelm (aducanumab) For the treatment of Alzheimer’s disease (AD)	Approved June 7, 2021	Medical benefit	<ul style="list-style-type: none"> High incidence* of this devastating disease (1.5 cases per 1,000 population) Questions about effectiveness noted by FDA advisory board and ICER May see high volume (patient, caregiver demand) First potentially disease modifying treatment High cost plus need for high volume of sites, specialists and costly tests could decimate budgets 	<ul style="list-style-type: none"> Prior authorization to ensure appropriate patient selection Evaluate options for clinically appropriate, least costly site of care
toripalimab For the treatment of nasopharyngeal (nose and throat) cancer	Anticipated approval, July 2021	Medical benefit	<ul style="list-style-type: none"> Although nasopharyngeal cancer is relatively rare, this drug also has fast-track status for mucosal melanoma (rare but very poor prognosis) and is showing promise in liver cancer (poor prognosis and more common at 1 case per 100,000 population). Cancer drugs are often used for non-approved indications. Current FDA designations - Breakthrough Therapy, Orphan Drug, Fast track 	<ul style="list-style-type: none"> Prior authorization to ensure use for FDA approved or compendia-endorsed indication(s) Evaluate options for clinically appropriate, least costly site of care

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TOP FIVE (continued):

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

WHAT <i>Drug & condition</i>	WHEN <i>FDA approval date</i>	WHERE <i>Probable benefit coverage</i>	WHY <i>What earned this drug a Top 5 placement</i>	ACTIONS <i>Strategies for managing cost</i>
<p>teplizumab</p> <p>For the prevention or delay of type 1 diabetes</p>	<p>Anticipated approval, July 2021</p>	<p>Medical benefit</p>	<ul style="list-style-type: none"> Prevention of destruction of insulin-producing cells would make this the first disease-modifying therapy ever for type 1 diabetes FDA Breakthrough Therapy and Priority Review designation About 5-10% of people with diabetes have type 1 diabetes, so its prevalence* is about 50 cases per 10,000 population 	<ul style="list-style-type: none"> Prior authorization to prevent use for type 2 diabetes and for appropriate patient selection Education
<p>reltecimod</p> <p>For organ dysfunction/ failure caused by “flesh-eating” bacteria (necrotizing soft tissue infections aka NSTIs)</p>	<p>Anticipated approval, September 2021</p>	<p>Medical benefit</p>	<ul style="list-style-type: none"> Novel mechanism: modulates the immune overreaction responsible for organ and tissue damage (not an antibiotic) Will not promote bacterial resistance Current FDA designations - Fast Track, Orphan Drug, Accelerated Approval 	<ul style="list-style-type: none"> Prior authorization to ensure organ dysfunction/ failure are present or suspected Complex case management

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RECENT FDA APPROVALS

Pharmacy Benefit

› **Empaveli (pegcetacoplan): Apellis**

- **Approval date: 5/14/2021**
- **Pharmacy benefit**
 - › Administered subcutaneously
 - › Available only through a Risk Evaluation and Mitigation Strategy (REMS) program
- **Indication and frequency**
 - › Paroxysmal nocturnal hemoglobinuria (PNH)(blood disease where immune cells attack red blood cells causing anemia)
 - › Prevalence*: 5,000-6,000 cases in the US
- **Cost factors**
 - › \$458,000 annually
 - › Therapeutic alternative annual treatment cost: Soliris (eculizumab) @ \$522K annually or Ultomiris (ravulizumab cwvz) @ \$459K
- **Therapeutic impact**
 - › Major advance
 - Fast Track, Priority Review, Orphan Drug
 - Unique mechanism that has shown significantly better results than Soliris in a head-to-head study; Ultomiris has a mechanism similar to Soliris
 - Carries a black box warning due to increased risk of infection
 - Low disease prevalence and competitive pricing suggest that impact on plan budgets will not be great
- **Management strategies**
 - › Inquire into PBM programs
 - › Prior authorization to confirm diagnosis
 - › Copay assistance may be available through ApellisAssist
 - › Home inventory management

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RECENT FDA APPROVALS (*continued*)

Pharmacy Benefit

> **Lumakras (sotorasib): Amgen**

- **Approval date: 5/28/2021**
- **Pharmacy benefit**
 - > Administered orally once daily
- **Indication and frequency**

Lung cancer (non-small cell lung cancer with KRAS G12C genetic mutation, locally advanced or metastatic, in patients who have tried at least one other systemic therapy): incidence* 8 cases per 100,000 population
- **Cost factors**
 - > Annual cost: \$217,774
 - > Therapeutic alternative annual treatment cost: Keytruda (pembrolizumab) + cisplatin + Alimta (pemetrexed) at \$227,000
- **Therapeutic impact**
 - > Major advance
 - FDA Real-Time Oncology Review pilot program and accelerated approval pathway
 - Continued approval contingent upon confirmatory trials for clinical benefit
 - First product to target this particular genetic mutation
 - Before this product, patients had poor prognosis, few treatment alternatives
- **Management strategies**
 - > Prior authorization to ensure trial/failure of first-line therapies and confirmation of KRAS G12C genetic mutation
 - > 10 week prior authorization approval duration
 - > Copay assistance may be available through Amgen First Step program
 - > Split fill program

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RECENT FDA APPROVALS *(continued)*

Pharmacy Benefit

› Truseltiq (infigratinib): BridgeBio Pharma, QED Therapeutics

- **Approval date:** 5/28/2021
- **Pharmacy benefit**
 - › Administered orally
- **Indication and frequency**
Often-fatal bile duct cancer (previously treated, unresectable cholangiocarcinoma with FGFR2 gene abnormalities); incidence* < 1 case per 100,000 population
- **Cost factors**
 - › \$280,265 annually
 - › Therapeutic alternative annual treatment cost: no real standard of therapy for this group of patients
- **Therapeutic impact**
 - › Incremental improvement
 - FDA Real-Time Oncology Review pilot program and accelerated approval pathway
 - Continued approval contingent upon confirmatory trials for clinical benefit
 - Approved based on a clinical trial where only 23% of patients responded, and only for a median duration of 5 months
- **Management strategies**
 - › Inquire into PBM programs
 - › Prior authorization to confirm FGFR2 gene abnormalities and unresectable (inoperable) tumor
 - › Copay assistance may be available through QED Therapeutics' ForgingBridges program

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RECENT FDA APPROVALS (*continued*)

Medical Benefit

> **Abecma (idecabtagene vicleucel): Bristol-Myers Squibb, Celgene, bluebird bio**

- **Approval date: 3/26/2021**

- **Medical benefit**

- > Given as a single lifetime IV infusion
- > Available only through a Risk Evaluation and Mitigation Strategy (REMS) program
- > Limited distribution

- **Indication and frequency**

Blood cancer (relapsed or refractory multiple myeloma, after 4 or more specific types of therapies); incidence* of myeloma, 1 case per 10,000 population

- **Cost factors**

- > One-time cost, \$419,500 (but requires 3 days of chemotherapy prior to infusion at a cost of approximately \$1400 total).
- > Therapeutic alternative annual treatment cost: many alternatives, ranging from \$170,000 to \$350,000 per year

- **Therapeutic impact**

- > Incremental improvement
 - There are numerous alternatives available for patients at this stage of therapy
 - Survival following administration was not as long as initially hoped.
 - Carries several FDA "black box warnings" about significant adverse effects

- **Management strategies**

- > Inquire into PBM programs
- > Prior authorization to confirm failure of previous therapies
- > Copay assistance may be available through CellTherapy360.com
- > Oncology case management
- > Investigate special arrangements such as centers of excellence/ certified healthcare facilities for administration

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RECENT FDA APPROVALS (*continued*)

Medical Benefit

> **Aduhelm (aducanumab): Biogen**

- **Approval date: 6/7/2021**

- **Medical benefit**

- > Administered as an IV infusion over 1 hour every 4 weeks

- **Indication and frequency**

- > Treatment of Alzheimer's disease: incidence* 1.5 cases per 1,000 population

- **Cost factors**

- > \$56,000 annually
- > Diagnostic testing requiring collection of cerebral spinal fluid, monitoring through PET scans, and infusion suites will be required (along with specialized staffing); with high volume of users, these could overwhelm national financial and medical provider resources.
- > The bulk of recipients will be Medicare beneficiaries.
- > Therapeutic alternative annual treatment cost: Not applicable; this is the first drug approved for this indication

- **Therapeutic impact**

- > Major advance (with caveats)
 - First approved product to treat the underlying cause of Alzheimer's disease
 - FDA accelerated approval; continued approval contingent upon confirmatory trials for clinical benefit
 - If the product demonstrates clinical benefits, it will be a boon to patients and their caregivers. FDA Advisory Committee, as well as many others, question whether the product provides a meaningful clinical benefit

- **Management strategies**

- > Prior authorization to confirm diagnosis
- > Evaluate options for clinically appropriate, least costly site of care

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RECENT FDA APPROVALS (*continued*)

Medical Benefit

> Jemperli (dostarlimab-gxly): GlaxoSmithKine

- **Approval date:** 4/22/2021

- **Medical benefit**

- > Administered intravenously over 30 minutes
- > Limited distribution

- **Indication and frequency**

Cancer of the uterus with a specific genetic defect (refractory or recurrent dMMR endometrial cancer that has progressed on or following preferred treatment): incidence* is about 6 cases per 100,000 population

- **Cost factors**

- > Annual cost: \$180,230
- > Therapeutic alternative annual treatment cost: Keytruda (pembrolizumab), \$177,660

- **Therapeutic impact**

- > Incremental improvement
 - Is the 7th product in its class; has not been directly compared to other treatments
 - FDA accelerated approval and Priority Review and Breakthrough Therapy designations; but continued approval contingent upon confirmatory trials for safety and efficacy.

- **Management strategies**

- > Inquire into PBM programs
- > Prior authorization to ensure trial/failure of first-line therapies and confirmation of dMMR cell type
- > Copay assistance may be available through GSK Copay Program
- > Oncology case management
- > Evaluate options for clinically appropriate, least costly site of care

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

> **Rybrevant (amivantamab-vmjw): Janssen**

- **Approval date: 5/21/2021**

- **Medical benefit**

- > Administered intravenously over 2-5 hours

- **Indication and frequency**

Lung cancer (metastatic and cisplatin-resistant non-small cell lung cancer (NSCLC) with epidermal growth factor receptor (EGFR) mutations): incidence* of NSCLC with this type of cell mutations: 1 case per 100,000 population

- **Cost factors**

- > Annual cost: \$233,582
- > Therapeutic alternative annual treatment cost: no suitable alternative

- **Therapeutic impact**

- > Major advance
 - First approved option for this subtype of NSCLC.
 - New mechanism of action for this subtype of NSCLC
 - Priority Review, Breakthrough Therapy
 - FDA accelerated approval; continued approval contingent upon confirmatory trials for safety and efficacy

- **Management strategies**

- > Inquire into PBM programs
- > PA to ensure test results confirm EGFR exon 20 insertion mutations, and that cancer has progressed while patient was on or after use of cisplatin
- > Copay assistance may be available through the Janssen CarePath Savings Program
- > Evaluate options for clinically appropriate, least costly site of care

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

> **Ryplazim (plasminogen, human-tvmh): Liminal BioScience**

- **Approval date:** 6/4/2021

- **Medical benefit**

- > Administered intravenously at 2-4 day intervals

- **Indication and frequency**

Enzyme deficiency that results in inflamed growths on mucous membranes and eyes; may cause blindness (congenital plasminogen deficiency): Prevalence is 1.6 cases per 1,000,000 population

- **Cost factors**

- > Annual cost: TBD
- > Therapeutic alternative annual treatment cost: no suitable alternative; current treatment is for symptoms only.

- **Therapeutic impact**

- > Major advance
 - Currently no established approach to treatment.
 - Orphan Drug, Rare Pediatric Disease Priority Review

- **Management strategies**

- > Inquire into PBM programs
- > PA to ensure appropriate diagnosis (may be sought for treatment of idiopathic pulmonary fibrosis) and to require testing to confirm increased trough plasminogen activity by an absolute 10% above baseline before approving renewals
- > Complex case management

RECENT FDA APPROVALS (*continued*)

Medical Benefit

› Zynlonta (fka Lonca)(loncastuximab tesirine-lpyl): ADC Therapeutics

- **Approval date:** 4/23/2021
- **Medical benefit**
 - › Administered intravenously over 30 minutes
- **Indication and frequency**
Blood cancer (relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy): incidence* 7.5 cases per 100,000 population
- **Cost factors**
 - › Annual cost: \$234,413
 - › Therapeutic alternative annual treatment cost: Yescarta (axicabtagene ciloleucel) or Kymriah (tisagenlecleucel) each at \$370,000 or Breyanzi (lisocabtagene maraleucel), \$400,000
- **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
 - FDA accelerated approval; continued approval contingent upon confirmatory trials for safety and efficacy
- **Management strategies**
 - › Inquire into PBM programs
 - › PA to ensure at least 2 lines of previous therapy; but given relative cost, criteria should not be more restrictive than those of Yescarta, Kymriah or Breyanzi
 - › Copay assistance may be available through ADVANCING Patient Support Copay Assistance Program
 - › 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
6/17/2021		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	<ul style="list-style-type: none"> Rare Pediatric Disease Orphan drug Breakthrough Therapy Fast-track 	TBD	No alternatives; symptomatic treatment only
2Q2021		tanezumab	Pharmacy--Subcutaneous	Osteoarthritis pain	32.5 million US adults, approximately 10% of US population	<ul style="list-style-type: none"> 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
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
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	N/A

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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
8/2021		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
9/2021		maralixibat	Pharmacy--Oral	Alagille syndrome (bile duct disorder that causes progressive liver damage)	Occurs in one case per 30,000 live births	<ul style="list-style-type: none"> Rare Disease Breakthrough Therapy Priority Review 	TBD	Ursodiol @ \$2.8K
10/2021		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
10/2021		somatrogon	Pharmacy--Subcutaneous	Pediatric growth hormone deficiency	Incidence* less than 1 case per 10,000 population	Compares to Genotropin/somatropin, but is dosed once weekly	TBD	Genotropin @ \$107K
3Q2021		abrocitinib	Pharmacy--Oral	Atopic dermatitis, moderate to severe (eczema; itchy skin rash)	Prevalence* of moderate to severe AD in the US: 11.7million	<ul style="list-style-type: none"> Breakthrough therapy Priority review Recent safety concerns with this drug class 	TBD	Dupixent @ \$42K

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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
4Q2021		pacritinib	Pharmacy--Oral	Myelofibrosis (bone marrow disorder that causes bleeding due to low platelet count)	Incidence* 1.5 cases per 100,000 population in the US	May have fewer side effects than other drugs in its class	TBD	Jakafi @180K
CRL 3/15/2021		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 results in more complete remissions after 3 years vs. Hydrea	TBD	Hydrea @ \$888

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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 in US (unless otherwise indicated)*	Comments	New Product Anticipated Annual Cost	Alternative Therapy on Market and Annual Cost
7/2021		narsoplimab	Medical-- Intravenous	Bone marrow/stem cell transplant-associated side effect: thrombotic microangiopathy (TM-TMA) (can cause anemia, bleeding, permanent organ damage or death)	Incidence* 1 case per 1,000,000 population		TBD	Defitelio @ \$170K
7/2021		teplizumab	Medical-- Intravenous	Prevention or delay of type 1 diabetes	Prevalence* of type 1 diabetes: 50 cases per 10,000 population	<ul style="list-style-type: none"> • Single treatment, likely over 14 days • Breakthrough Therapy • Priority Review 	TBD	No alternatives; symptomatic treatment only
7/2021		toripalimab	Medical-- Intravenous	Nasopharyngeal (nose and throat) cancer	Incidence* of < 1 case per 100,000 population	<ul style="list-style-type: none"> • Breakthrough Therapy • Orphan Drug • Fast track 	TBD	Cisplatin + Gemcitabine @ \$24K
8/2021		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

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ANTICIPATED FDA APPROVALS *(continued)*

Medical 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
8/2021		vicineum	Medical-- Administered into the bladder	Resistant bladder cancer	Incidence* 2 cases per 10,000 population		TBD	<ul style="list-style-type: none"> Keytruda @ \$150K or gemcitabine @ \$12K
9/2021		reltecimod	Medical-- Intravenous	Organ dysfunction/ failure caused by "flesh-eating" bacteria (necrotizing soft tissue infections aka NSTIs)	Incidence* of approximately 10 cases per 100,000 population	<ul style="list-style-type: none"> Novel mechanism Fast Track Accelerated Approval 	TBD	No alternatives specifically approved for NSTIs; note that NSTIs often require limb amputation
10/2021		tisotumab vedotin	Medical-- Intravenous	Metastatic or recurrent cervical cancer (type of uterine cancer)	Incidence* of cervical cancer: 4 cases per 100,000 population	<ul style="list-style-type: none"> Accelerated Approval Priority Review 	TBD	Pembrolizumab @ \$175K
12/2021		efgartigimod	Medical-- Intravenous	Myasthenia gravis (nervous system disorder)	Incidence* < 1 case per 100,000 lives	<ul style="list-style-type: none"> Orphan drug Early access program allows use prior to FDA approval. 	TBD	Soliris @ \$700K

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ANTICIPATED FDA APPROVALS (continued)
Medical 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
2021		ciltacabtagene autoleucl	Medical-- Intravenous	Multiple myeloma (type of blood cancer)	Incidence* is 1 case per 10,000 population	<ul style="list-style-type: none"> Rare Disease Breakthrough Therapy 	TBD	<ul style="list-style-type: none"> Pepaxto @ \$206K Combination regimens \$169K-\$355K Blenrep @ \$246K
2021	Lantidra	donislecl	Medical-- transplant into Portal vein	Diabetes: Labile (aka brittle) Type 1 diabetes not well controlled with intensive insulin therapy	Prevalence* of labile type 1 diabetes is 2 cases per 10,000 population	FDA Advisory Committee concluded it has "overall favorable benefit-risk profile" for certain people	TBD	Pancreas transplant @ \$408K

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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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