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4TH 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*
Korsuva (difelikefalin) Treatment of itching associated with chronic kidney disease (CKD)	Approved August 23, 2021	Medical benefit	• First FDA approved treatment for itching associated with CKD • Priority review, Breakthrough therapy	• Prior authorization to ensure appropriate patient selection
Rezurock (belumosudil) Treatment of chronic graft vs host disease (cGVHD)	Approved July 16, 2021	Pharmacy benefit	• Only product with this mechanism of action for this condition • First approved cGVHD treatment for children • Priority Review, Breakthrough Therapy designation	• Prior authorization to confirm patient is an appropriate candidate for therapy • Home inventory management
Welireg (belzutifan) Treatment for patients with cancer due to von Hippel-Lindau disease.	Approved August 13, 2021	Pharmacy benefit	• Offers a treatment option for patients with tumors from von Hippel-Lindau disease who are not candidates for surgery • Significant overall response rates for a variety of VHL-associated cancers	• Prior authorization to confirm VHL alteration and patient is not a candidate for surgery • Copay assistance may be available through Merck & Co. • Split fill program
ganaxolone Treatment of patients with CDKL5 deficiency	Anticipated approval in March 2022	Pharmacy benefit	• Would be the only therapy specifically for this condition • Current treatment is not effective at stopping seizures in many patients • Priority Review designation	• Prior authorization to confirm clinical need for the product • Consider referral to complex case management program.
efgartigimod For the treatment of myasthenia gravis	Anticipated approval 4th quarter 2021	Medical benefit	• Offers additional treatment option for patients who cannot use conventional therapies • Price has not been established but is anticipated that it will be less expensive than Soliris	• Prior authorization to ensure appropriate patient selection • Evaluate options for clinically appropriate, least costly site of care

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RECENT FDA APPROVALS

Pharmacy Benefit

Bylvay (odevixibat): Albireo

- **Approval date: 7/20/2021**
- **Pharmacy benefit**
  - Administered orally once daily
  - Limited distribution
- **Indication and frequency**
  - Treatment of itching associated with progressive familial intrahepatic cholestasis: prevalence* 1-2 cases per population of 100,000
- **Cost factors**
  - $321,200 annually
  - Annual cost estimate based on average weight of 18kg for patients in clinical
  - Therapeutic alternative annual treatment cost: Not applicable; this is the first drug approved for this indication
- **Therapeutic impact**
  - Major advance (with caveats)
    - First product to treat itching associated with progressive familial intrahepatic cholestasis
    - May not be effective for patients with subtype 2 disease
- **Management strategies**
  - Prior authorization to confirm patient is an appropriate candidate for therapy
  - Copay assistance may be available through Albireo

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RECENT FDA APPROVALS

Pharmacy Benefit

Wegovy (semaglutide): Novo Nordisk

- **Approval date: 6/04/2021**
- **Pharmacy benefit**
  - Administered subcutaneously once weekly
- **Indication and frequency**
  Treatment of obesity, or overweight in the presence of at least one weight-related comorbidity: prevalence* approximately 40-45 cases per population of 100
- **Cost factors**
  - $16,188 annually
  - Therapeutic alternative annual treatment cost: Saxenda (liraglutide) $16,188 annually
- **Therapeutic impact**
  - Incremental improvement
    - Offers significant improvement in weight-loss when indirectly compared to similar agent in class
- **Management strategies**
  - Prior authorization to ensure appropriate use and reauthorization only when benefit documented

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RECENT FDA APPROVALS *(continued)*

Pharmacy Benefit

Rezurock (belumosudil): Kadmon

- **Approval date: 7/16/2021**
- **Pharmacy benefit**
  - Administered orally once daily
  - Limited distribution
- **Indication and frequency**
  Treatment of chronic graft-versus-host disease (cGVHD)(bone marrow/stem cell transplant side effect that can be fatal): incidence* 1-1.5 cases per population of 100,000 per year
- **Cost factors**
  - Annual cost: $188,583
  - Therapeutic alternative annual treatment cost: Imbruvica (ibrutinib) at $181,529
- **Therapeutic impact**
  - Major advance
    - First FDA-approved product with this mechanism of action for the treatment of chronic GVHD; first FDA-approved drug for cGVHD for patients 12-18 years of age
    - Received Priority Review and Breakthrough Therapy designation
    - Treatment likely to be reserved for patients who fail or are not candidates for Imbruvica
- **Management strategies**
  - Prior authorization to confirm patient is an appropriate candidate for therapy
  - Home inventory management
  - Copay assistance may be available through Kadmon

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RECENT FDA APPROVALS *(continued)*

Pharmacy Benefit

Skytrofa (lonapegsomatropin): Ascendis

- **Approval date: 8/25/2021**
- **Pharmacy benefit**
  - Administered as subcutaneous injection weekly
- **Indication and frequency**
  Treatment of pediatric growth hormone deficiency; prevalence* 1 to 2.5 cases per population of 10,000
- **Cost factors**
  - Cost currently unavailable
  - Therapeutic alternative annual treatment cost: Numerous treatments approved, including Norditropin (somatropin) at $65,461, Humatrope (somatropin) at $65,838, Omnitrope (somatropin) at $58,522
- **Major advance (with caveats)**
  - Offers once weekly dosing
  - May offer a slight improvement in effectiveness
  - Annual price is uncertain but likely to be at premium to daily-dosed products
- **Management strategies**
  - Prior authorization to ensure patient is an appropriate candidate
  - Inventory management

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RECENT FDA APPROVALS *(continued)*

Pharamcy Benefit

Welireg (belzutifan): Merck & Co

- **Approval date:8/13/2021**
- **Pharmacy benefit**
  - Administered orally
- **Indication and frequency**
  Adult patients with von Hippel-Lindau (VHL) disease who require systemic treatment for associated kidney, nervous system and pancreatic tumors; prevalence* 2.7 cases per population of 100,000
- **Cost factors**
  - $267,500
  - Therapeutic alternative annual treatment cost: not applicable, no real standard of therapy for this group of patients.
- **Therapeutic impact**
  - Major advance
    - Offers effective treatment option for patients with von Hippel-Lindau disease who are not candidates for surgical intervention
    - Study population demonstrated significant overall response rates for a variety of VHL associated cancers
- **Management strategies**
  - Prior authorization to confirm VHL alteration and patient is not a candidate for surgery
  - Copay assistance may be available through Merck & Co
  - Split fill program

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Korsuva (difelikefalin): Cara Therapeutics, Vifor Pharma

- **Approval date:8/23/2021**
- **Medical benefit**
  - Administered as IV infusion 3 times weekly following dialysis
- **Indication and frequency**
  Treatment of moderate to severe itching associated with chronic kidney disease in adults on dialysis; prevalence* of 70 cases per population of 100,000
- **Cost factors**
  - $14,1666
  - Therapeutic alternative annual treatment cost: Not applicable; this is the first drug approved for this indication
- **Therapeutic impact**
  - Major advance
    - First FDA approved product for moderate-to-severe itching associated with CKD in adults receiving hemodialysis
    - Received Priority Review and Breakthrough Therapy designation
- **Management strategies**
  - Prior authorization to confirm patient is an appropriate candidate for therapy (e.g. patient has failed conventional therapy)
  - Copay assistance may be available through Cara Therapeutics or Vifor Pharma

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RECENT FDA APPROVALS *(continued)*

Medical Benefit

Nexviazyme (avalglucosidase alfa-ngpt): Sanofi, Genzyme

- **Approval date:6/7/2021**
- **Medical benefit**
  - Administered as an IV infusion over ~7 hour every 2 weeks
  - Weight based dosing
- **Indication and frequency**
  Treatment of late-onset Pompe disease (rare genetic enzyme deficiency that damages liver and heart); prevalence* 2.5 cases per population of 100,000
- **Cost factors**
  - Annual cost: $713,398
  - Therapeutic alternative annual treatment cost: Lumizyme (alglucosidase alfa) at $691,105
  - Annual cost estimates based on 76 kg patient
- **Therapeutic impact**
  - Incremental improvement
    - Second enzyme replacement therapy (ERT) for late-onset Pompe disease available in US
    - Differs from Lumizyme in that Nexviazyme targets key pathway for cellular uptake of enzyme replacement
    - Treatment effectiveness appears similar to Lumizyme
- **Management strategies**
  - Prior authorization to confirm diagnosis
  - Evaluate options for clinically appropriate, least costly site of care
  - Copay assistance may be available through Sanofi, Genzyme

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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
3/2022		ganaxolone	Pharmacy--Oral	CDKL5 deficiency (causes severe, frequent seizures that are difficult to treat)	2-3 cases per 100,000 live births	• Rare Disease and Priority Review designations • Likely to be high since no other specific treatment	TBD	N/A: currently no curative or specific therapies available for CDD
2/2022		bardoxolone	Pharmacy--Oral	Alport Syndrome (may cause loss of kidney function, deafness and more)	Prevalence*: 1 case per population of 5-10,000	• Rare Disease and Priority Review designations • Likely to be high since no other effective treatment	TBD	N/A: no existing drug therapy considered effective
1/2022		carbetocin	Pharmacy--Nasal	Prader-Willi syndrome (can cause distress, extreme overeating, other effects)	Prevalence*: 1 case per population of 25,000	• Rare Disease; Fast Track; Priority Review designations • Different mechanism of action than the only alternative	TBD	Norditropin, Humatrope, etc.) @ approximately $100K
2/2022		mitapivat sulfate	Pharmacy--Oral	Pyruvate kinase deficiency (PKD); causes mild to severe anemia, sometimes other serious effects.	Incidence*: Unknown; mild cases probably undiagnosed. Approximately 1 case per 1 million population is diagnosed.	• Orphan drug, Priority Review, Rare Disease; Fast Track • First potentially disease-modifying therapy for this condition	TBD	N/A: No existing drug therapy. Treatment includes blood transfusions, spleen removal, and rarely, stem cell transplantation

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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
10/2021		avacopan	Pharmacy--Oral	ANCA associated vasculitis (rare disorder of blood vessels causing kidney and lung problems)	Prevalence*: < 1 case per population of 10,000	Unique mechanism of action	TBD	Rituxan @ $19K
11/2021	Voxzogo	vosoritide	Pharmacy--Subcutaneous	Achondroplasia, a rare, genetic type of dwarfism	Incidence*: 3-5 cases per 100,000 live births	First product to treat condition	$300,000	N/A--no existing treatment
9/2021	Livmarli	maralixibat	Pharmacy--Oral	Alagille syndrome (bile duct disorder that causes progressive liver damage)	Occurs in one case per 30,000 live births	• Rare Disease • Breakthrough Therapy • Priority Review	TBD	Ursodiol @ $2.8K
10/2021		bimekizumab	Pharmacy--Subcutaneous	Plaque psoriasis (common skin condition)	Prevalence*: 8 cases per population of 10,000	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 population of 10,000	Compares to Genotropin/somatropin, but is dosed once weekly.	TBD	Genotropin @ $107K

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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
TBD (FDA announced in July that 7/27 PDUFA date would not be met)		abrocitinib	Pharmacy--Oral	Atopic dermatitis, moderate to severe (eczema; itchy skin rash)	Prevalence* of moderate to severe AD in the US: 3.5 cases per population of 100	• Breakthrough therapy • Priority review • Recent safety concerns with this drug class	TBD	Dupixent @ $42K
4Q2021		pacritinib	Pharmacy--Oral	Myelofibrosis (bone marrow disorder that causes bleeding due to low platelet count)	Prevalence*: 1.5 cases per population of 100,000	May have fewer side effects than other drugs in its class		Jakafi @180K
CRL** issued 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 population of 100,000	Early clinical trials show ropeginterferon alfa-2b results in more complete remissions after 3 years than Hydrea	TBD	Hydrea @ $888
CRL** issued 6/18/2021		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	• Rare Pediatric Disease • Orphan drug • Breakthrough Therapy • Fast-track	TBD	N/A: No alternatives; symptomatic treatment only

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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
3/2022		ublituximab	Medical--Intravenous	Chronic lymphocytic leukemia (slow-growing form of blood cancer	Prevalence*: 6 cases per population of 100,000	Priority Review designation	TBD	Zydelig + rituximab @$125K per 6 months
3/2022		relatlimab + nivolumab	Medical--Intravenous	Melanoma (type of skin cancer) that has spread or cannot be surgically removed	Prevalence*: 3 cases per population of 10,000	Priority Review designation	TBD	Yervoy + Opdivo @ $138K per 3 months.
2/2022		tebentafusp	Medical--Intravenous	Uveal melanoma (cancer in the eye), certain subtypes	Prevalence*: 5 cases per population of 1 million	Priority Review, Breakthrough Therapy, Real-Time Oncology pilot program designations	TBD	Keytruda @ $122K
3/2022	Tyvyt	sintilimab	Medical--Intravenous	Non-small cell lung cancer, non-squamous cell subtype	Prevalence*: 4 cases per population of 10,000		TBD	N/A: sintilimab is being reviewed as an add-on to the existing standard of care
10/2021		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 population of 1,000,000 per year		TBD	Defitelio @ $170K

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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
7/2021	Tuoyi	toripalimab	Medical--Intravenous	Nasopharyngeal (nose and throat) cancer	Prevalence*: < 1 case per population of 100,000	Breakthrough Therapy, Orphan Drug, Fast track designations	TBD	Cisplatin + Gemcitabine @ $24K
9/2021		reltecimod	Medical--Intravenous	Organ dysfunction/ failure caused by "flesh-eating" bacteria (necrotizing soft tissue infections aka NSTIs)	Incidence* of approximately 1.5-4.5 cases per population of 1 million per year	• Novel mechanism • Fast Track, Accelerated Approval designations • High impact: NSTIs often require limb amputation	TBD	N/A: No alternatives specifically approved for NSTIs
10/2021	Tivdak	tisotumab vedotin	Medical--Intravenous	Metastatic or recurrent cervical cancer (type of uterine cancer)	Prevalence* of cervical cancer: 4 cases per population of 100,000	Accelerated Approval, Priority Review designations	$400,000	Pembrolizumab @ $175K
12/2021		efgartigimod	Medical--Intravenous	Myasthenia gravis (nervous system disorder)	Prevalence* < 1 case per population of 100,000	• Orphan drug • Early access program allows use prior to FDA approval.	TBD	Soliris @ $700K

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Medical Benefit

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11/2021		ciltacabtagene autoleucel (cilta-cel)	Medical--Intravenous	Multiple myeloma (type of blood cancer)	Prevalence* is 1 case per population of 10,000	Rare Disease, Breakthrough Therapy designations	TBD	• Pepaxto @ $206K • Combination regimens $169K-$355K • Blenrep @ $246K
2021	Lantidra	donislecel	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 population of 10,000	FDA Advisory Committee concluded it has "overall favorable benefit-risk profile" for certain people		Pancreas transplant @ $408K
CRL** issued 7/2/2021		teplizumab	Medical--Intravenous	Prevention or delay of type 1 diabetes	Prevalence* of type 1 diabetes: 50 cases per population of 10,000	• Single treatment, likely over 14 days • Breakthrough Therapy, Priority Review designations	TBD	No alternatives; symptomatic treatment only
CRL** issued 8/13/2021	Vicineum	oportuzumab monatox-qqrs	Medical--Administered into the bladder	Resistant bladder cancer	Prevalence* 2 cases per population of 10,000	Priority Review designation	TBD	• Keytruda @ $150K • Gemcitabine @ $12K

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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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