Canadian Organization for Rare Disorders

February 14, 2020

VIA EMAIL: ***PMPRB.Consultations.CEPMB@pmprb-cepmb.gc.ca***

Patented Medicine Prices Review Board (PMPRB)
333 Laurier Avenue West, Suite 1400
Ottawa, Ontario K1P 1C1

Dear Members of the PMPRB,

We cannot state strongly enough the expressions of terror, betrayal and utter disbelief we have heard from Canadians living with rare diseases as they learn about the changes to the Patented Medicines Regulations and proposed implementation guidelines. As the national association of 100+ rare disease patient organizations, the Canadian Organization for Rare Disorders (CORD) represents nearly 3 million Canadians affected by rare diseases. Members come to us seeking rationale, justification, and clarification of these seemingly ill-founded and inevitably calamitous guidelines. Sadly, we have to admit that despite our conscientious engagement over the past two-to-three years, we have not been provided with the research, evidence, or expert opinion to support their claim that proposed actions will save substantial drug expenditures while not harming patient access to the most appropriate medicines.

In response to previous "consultations", CORD has provided feedback on the most salient actions. We reiterate key points.

- It is reasonable to test the impact on drug expenditures of switching from seven to 11 (different) countries as the reference for a new drug's maximum list price
- It is ridiculous to introduce a cost-effectiveness assessment (CEA) at the time of launch to set a legally binding rebated price. If a CEA is calculated, it should inform the price negotiation process, which is how it is used currently and in every other country in our reference list. For most novel or innovative drugs (including most of those for rare diseases), the level of uncertainty in conducting a CEA/HTA makes the exercise moot.
- It is even more outrageous to adopt a universal (legally binding) "value" index, namely an ICER of $60k/QALY for drugs for common conditions or $90k/QALY for a drug intended for a rare disease population. While some other jurisdictions have set either formal or informal ICER, no one has set these as legal limits. It would preclude consideration of many other factors, including severity of disease, other treatment options, challenges of administration, and, indeed, leaves no room for negotiation.

These guidelines are inherently biased against drugs for small, difficult-to-diagnose and previously untreated patient populations with poorly documented natural history of disease and little clinical evidence of direct links between biomarkers and other outcome measures. This description would apply to almost all rare diseases, so these "universal" guidelines and pricing standards inexorably discriminate against those with rare conditions.

We wish to point out specifically the tremendous harm that these proposed guidelines will inevitably inflict on Canada's rare disease community. The discovery of a potentially beneficial medicine offers not only hope but also the first real chance of managing painful and debilitating symptoms, halt disease progression, and even avoid premature death. In most cases, this novel drug will be the first effective treatment developed for that specific disease. We cannot overstate

the fact that rare disease patients are heavily dependent on the discovery and introduction of innovative, new medicines for survival and well-being. The proposed PMPRB guidelines will severely deter setting up clinical trials, timely introduction of new medicines, investment in research, clinics, and patient support programs, and establishment of diagnostic and treatment protocols. In short, Canada will fall far behind best practices and treatment standards of most developed countries.

Since the changes to the Patented Medicines Regulations were first proposed in 2017, we have consistently raised concerns and put forward alternative proposals, based on best practices in other countries, that can address optimizing drug budgets and assuring appropriate access and use of medicines, toward a cost-effective national drug strategy.

To be frank, we have little expectation that these comments and recommendations will make much difference to the guidelines that have changed "almost not at all" since the initial proposal in 2017 and the latest draft shared December 9-10, 2019. Indeed, we, along with other patient organizations, have been denigrated and diminished in the Steering Committee and in public, have had our expertise and integrity questioned in closed sessions as well as in public consultations, and have been denied access to important background documents and opinions commissioned by the PMPRB.

This lack of responsiveness to input is particularly problematic given the "none-to-scant" evidence, rationale, testing, and/or plain old common sense evident in the previous drafts of these guidelines. Additionally, neither David Dodge nor the PMPRB-appointed Technical Working Group could or would validate the "numbers" in the draft guidelines. I was not alone as a member of the Steering Committee in raising concerns about the lack of research substantiating the models, the arbitrary and unreasonable "point values" of $60k and $90k per QALY for common and rare disease drugs, respectively.

CORD was not alone in raising these alarm bells: hundreds of stakeholders have highlighted these concerns. The PMPRB's own technical working group highlighted issues from the experts who were supposed to help Staff determine a workable framework. Below are just a few verbatim comments from that report, which need to be considered when reviewing the guidelines in the current consultation:

- "… provide an incentive for manufacturers to avoid launching in one or more indications"
- "If the reduction in ceiling price for medicines with large market size is large, then manufacturers may be incentivized to reduce the quantity supplied so as to avoid the reduction in the ceiling price"
- "Concern was raised that this approach would provide a disincentive for manufacturers to conduct research that reduces uncertainty around the ICER, since additional uncertainty would be rewarded with a higher ceiling price"
- **"Under such uncertain circumstances, it is foreseeable that many companies will delay or even forgo the launching of new innovative medicines in Canada"**

Having already commented extensively in written consultations, in the meetings of the Steering Committee on Guidelines, in meetings with PMPRB Staff and in the recent Civil Society Forum, we do not need to repeat the reasons why the proposed approach will have an impact on access to medicines. We simply have to point you, as members of the Board, to three facts on the ground:

1. Medicines are not coming to Canada as a result of unnecessary pricing uncertainty, denying access to medicines that can cure blindness, improve mobility and restore the ability to breathe

2. Our members who have access to rare disease treatments recognize that if these new rules were in place when their therapy was coming to market, they would never have been able to be treated

3. The thousands of patients with rare diseases that don't have access to medicines today but can see new technologies under development will have no access to clinical trials and experimental therapies

1. Medicines are not coming to Canada

You don't have to wait for one full year after implementation to consider the impacts of the PMPRB changes. The impacts are happening today.

Since October 2019, shortly after the adoption of the new rules, 21 medicines were approved by the US FDA, and NONE of them have been submitted to Health Canada (see attached table). While we cannot blame all of these non-applications on the changes to PMPRB, we have spoken directly to some of the Canadian and global representatives of these manufacturers who have stated that, despite their best attempts, they cannot get agreement from the global office to include Canada in the first tier. We have heard clearly that companies are already starting to deprioritize the Canadian market for new medicine launches because they cannot afford the impact on global pricing. We can't blame them.

In a review comparing CADTH recommendations for designated orphan drugs with those of Quebec, Australia, Scotland and New Zealand, they found similar rates of positive recommendations for the period January 2004 to October 2017. Importantly, positive recommendations were given for 62.5% of drugs with ICER's over $100k per QALY. Specifically, three of these are life-saving/life-altering therapies for hypophosphatasia, mucopolysaccharidosis IVA, and systemic idiopathic juvenile arthritis with ICER's at $2.7 million/QALY, $1.72 million/QALY, and 824k/QALY, respectively.[1] Even with negotiated price reductions, it is obvious that none of these therapies would have met the proposed PMPRB threshold and, indeed, would probably have never been submitted for access in Canada.

[1] https://ojrd.biomedcentral.com/track/pdf/10.1186/s13023-018-0759-9

New research presented to us suggests that average price reductions for rare disease medicines will be 82.5%.[2] This is much greater than PMPRB originally estimated to use. This means that patients will have to wait additional years to access to the medicines they need, and many patients simply can't afford to wait.

For instance, Trikafta, a new treatment for cystic fibrosis which can help patients who have the most common gene mutation that causes the disease, was just approved by the FDA in October 2019. This one drug alone has so much potential to help the majority of cystic fibrosis patients who previously had no other options to live longer and in better health. But due to the uncertain pricing environment in Canada, the chances of this treatment being made available in Canada in the near future are slim.

Even when companies do decide to file new drug submissions with Health Canada, many of these life-saving medicines are not being submitted to CADTH and INESSS. We spoke with another major company that has just received Health Canada NOC for a drug for a very small patient population but will not submit to the public drug programs. This means that even if the drug is available for sale, patients who depend on public drug programs will be denied access. In the meantime, we are working with them to see if there is the possibility for a compassionate access program sponsored by the company, since the condition is otherwise terminal. Sadly, Canada is spiraling downward to the level of New Zealand, where most rare disease patients do not have access to treatments available in most other developed countries and where they do, it comes from company-sponsored charitable donations. Unfortunately, with a population that is more than seven times that of New Zealand, Canada cannot hope that companies will treat us with the same compassion…nor should they.

We have asked the PMPRB to conduct impact analyses and to prove their contention that the proposed guidelines will have no effect on patient access to novel therapies. acannot not wait for one year after the changes have come into effect to assess impacts on access

2. Currently available rare disease treatments would not be available if the proposed Guidelines had been in place in recent years

Statistics don't tell even a fraction of the story that you need to hear, and which CORD and our members can contribute. We have received and have been made aware of dozens of situations where patients are fearful of not having access to the next generation therapy, or who value what they are currently benefitting from and fear that it will not be available going forward. We have included just two of those stories below for you to consider.

Patient story 1:

> *My name is Tina McGrath & I have Hereditary Angioedema (HAE) which affects 1-10,000 to 50,000 people.*

[2] PDCI; February 12, 2020; Impact Analysis Of The Draft PMPRB Excessive Price Guidelines: http://www.pdci.ca/wp-content/uploads/2020/02/PDCI-PMPRB-Impact-Assessment-February-2020_Final.pdf

My first memory of any issues was when I was 10. At 15, I had 6 unnecessary Laparoscopic surgeries and had my healthy appendix removed. Before being diagnosed, I had seen 10+ different physicians and specialists. I felt disabled by symptoms I could not control. It was 18 years later I was finally diagnosed with HAE by an allergist/immunologist, but he was unsure of treatments. Throughout the years my symptoms changed from predominately belly swells and abdominal pain to my hands, feet, face and throat swelling ~ 4-5 times a week. All of this came with extreme exhaustion.

I had tried several treatments without much relief. I struggled throughout the years with poor quality of life. As a student I missed many days of school. As an adult I missed work due to HAE, but felt the need, when possible, to work through the symptoms, for fear of losing my job. I missed many important events in my life, due to being admitted into the hospital. I have had a throat swell so bad that the Dr. wanted to put in a tracheotomy.

Life with HAE is always unpredictable.

I'm happy to say that the last 7 years, I have been home infusing which has my symptoms managed. Without access to treatment, I believe I would have NO quality of life and would be spending a lot of time at the Hospital emergency seeking treatment.

Patient story 2:

I was born with Cystic Fibrosis a rare genetic disorder that affects all of the organs, with most damage being done to lungs and digestive system. Despite living on handfuls of pills for my digestion, spending hours doing masks and therapy for my lungs, I would end up in hospital for two or three week admissions twice a year to fight the constant infection in my lungs. My quality of life as a child was pretty sad, I never felt good. Chronic sinus infections that left me with headaches, digestive problems that gave me stomach pain that would never go away and I was tired ALL the time. I was always away from school, because of this I had problem with friends as I couldn't attend a lot of the activities my friends went to as I was too sick. It was scary and as I approached my 11th birthday things were only getting worse. I was very tiny, below the 5th percentile in both height and weight, my lung function was in the 70's despite semi annual two week admissions to Sick Kids to fight infection.

I was trying so hard to do what I could to get healthy and live the fun lives my friends were. I raised money with my family to help find new treatments for CF and I gave blood and often spent days in hospital participating in research to help bring these new drugs and treatments for our CF Community. Well I struck it lucky when I enrolled to do a blind study for a new drug called Kalydeco, a gene modifier for my specific rare form of CF. My lung function jumped by 30% in only 30 days, my weight increased by nearly 15lbs, more weight than I had been able to gain in years and for the first time that I could remember I could breathe through my nose. This was the real deal, I couldn't remember ever feeling this good. Now this is a pretty pricey drug and it was not easy for me to gain access to this

either. I had done everything I could to get this drug including going to Queens Park to explain how important this drug was to me and others in the same position as me.

I have been living an amazing life since gaining access to that drug, including proudly being a Sick Kids Ambassador and Global Ambassador for a Youth Group for Disabilities, finishing high school, working, volunteering, advocating for others who also need lifesaving medication. This past September I proudly walked 5 days on the Greta Wall of China raising money and awareness for CF. My life matters, these drugs gave me my life. Please don't steal lives from people who have spent there lives fighting to live.

Patient story 3:

Family races to raise $2.8M to treat two-month-old baby with rare muscular disease

Two-month-old Eva Batista is struggling with spinal muscular atrophy, a deadly neuromuscular disorder that results in progressive muscle wasting and the loss of motor neurons, and the only possible onetime treatment, worth millions of dollars, is not available in Canada. Her parents, Ricardo Batista and Jessica Sousa, from North York, say they are doing everything they can to raise the $2.8 million needed to take their daughter to the U.S. and pay for Zolgensma, a onetime treatment and the most expensive drug in the world.

Batista told CTV News Toronto that their baby started her first treatment of a drug called Spinraza, which was just approved in Canada in 2016. He said the drug, which is covered in Ontario for her, will extend her life for an undetermined amount of time by slowing down the progression of the disease, but it is not a one-time treatment or cure.[3]

Canadian parents of babies with rare deadly disease look to Novartis treatment lottery

The Swiss pharmaceutical company that makes Zolgensma, a US$2.1-million gene therapy, is planning to give away as many as 100 doses of the one-time treatment this year in countries where the drug is not yet approved, including Canada.

Novartis began accepting applications for the lottery, which it calls a "managed access program," on Jan. 2. The company intends to select one baby or toddler with spinal muscular atrophy (SMA) at random every two weeks.

Yet some bioethicists say that in a scenario such as this one – where all babies with SMA are equally medically needy and the company says it does not have the production capacity to give away more than a limited supply – a lottery may be the best of the bad options for divvying up free Zolgensma.

Despite his queasiness about the concept, Mr. Batista said he and his wife, Jessica, will likely put Eva's name forward for the lottery. Time is not on their daughter's side.[4]

[3] https://toronto.ctvnews.ca/family-races-to-raise-2-8m-to-treat-two-month-old-baby-with-rare-muscular-disease-1.4652225

[4] https://www.theglobeandmail.com/canada/article-parents-of-babies-with-deadly-rare-disease-desperate-to-win-drug-maker/

The fact that Eva is alive and in good enough condition to be considered for Zolgensma is the result of a number of "miracles." Spinraza was approved by Health Canada in July 2017 and received a positive recommendation to reimburse from CADTH in November 2017. It was approved for infants with SMA-Type 1 under the age of 7 months in November 2018 in Ontario.

The fact that CADTH made a positive recommendation to fund and an agreement was subsequently negotiated through pCPA and accepted by provinces is a minor miracle. The manufacturer reported ICURs for nusinersen compared with RWC as follows: for SMA type I, $665,570 per QALY; for SMA type II, $2.1 million per QALY; and, for SMA type III, $2.9 million per QALY. The manufacturer indicated the probability that nusinersen was cost-effective assuming a willingness to pay of $300,000 per QALY was 0% for all SMA types.

CDR reanalysis reported much higher incremental costs per QALY estimates: $9.2 million for SMA type I and $24.4 million for SMA type II. Results for SMA type III should be considered speculative, given the concerns raised due to the lack of appropriate clinical data. Analysis based on the limited data available concluded nusinersen was unlikely to be cost-effective with an incremental cost per QALY of $7.4 million for SMA type III. For each SMA type, the probability that nusinersen was cost-effective at a willingness-to-pay threshold of $500,000 remained 0%.[5]

The initial recommendation was to reimburse only for SMA type I patients under 7 months of age. Upon resubmission with additional clinical trial data, CADTH recommended treatment for patients up to 12 years of age without ability to walk.[6]

If Eva had been born 18 months earlier, she may not have received the Spinraza that has allowed her family to fight for Zolgensma. is important to note that CADTH making a positive recommendation at ICERs that were, by their own estimates, in the range of $9.2 and $24.4 million for SMA type I and type II, respectively. If the company had brought Spinraza to market after the revised PMPRB guidelines were implemented (July 2020), no Canadian baby with SMA would likely have received Spinraza. There is no way that it could meet the threshold of $90k/QALY. It is unclear as to which agency the company could negotiate with if the $90k/QALY is deemed legally binding.

Patient story 4:

Joseph is a 37-year-old living with sickle cell disease. Instead of flexible, round red blood cells, Joseph's cells are rigid, crescent-shaped, and sticky. They can easily get stuck in small blood vessels, slowing or blocking blood flow and oxygen to parts of the body. As a result, Joseph experiences extreme fatigue, breathlessness, and other symptoms of anemia. Much worst are the periodic episodes of pain, called pain crises, that can vary in intensity and last for a few hours to a few weeks, sometime necessitating hospitalization. Finally, SCD puts Joseph at risk for frequent infections. As evident, persons living with SCD must develop significant resilience and resolution to carry out work, school, and other daily activities.

[5] https://cadth.ca/sites/default/files/cdr/complete/SR0525_Spinraza_complete_Dec_22_17.pdf

[6] https://cadth.ca/sites/default/files/cdr/complete/SR0576-Spinraza-Resubmission-Mar-1-19.pdf

For decades, there have been no effective treatments for SCD, only symptom management and supportive care, including antibiotics, hydroxyurea, and pain killers. In November 2019, the FDA approved Adakveo, the first therapy that can prevent pain crises. Actually, a second therapy, Obryta, that can prevent the red blood cell deformation, showed such dramatic results in early clinical trials that the FDA granted breakthrough status, accelerated approval and early market access three months in advance of the projected approval date.

Neither drug has been submitted to Health Canada and there are no immediate plans to file, despite the pleas from the Canadian sickle cell community. An earlier therapy, Endari, which has been shown to significantly reduce the incidence of pain crises in children, is in review by Health Canada. We do not have Canadian ICER's for any of these therapies; however, ICER in the USA conducted HTA reviews for all three and estimated $/QALYs between $150k to $1 million per QALY, which is not atypical for innovative drugs but obviously exceeding the proposed PMPRB threshold of $60k or even $90k.

So, just at a time when effective therapies are finally available for the sickle cell patient, Canada is setting up significant deterrents for companies to bring these therapies to Canada.

3. Research / clinical trials

Clinical trials are a vital component of rare disease treatment in Canada and are often lifelines for patients who would otherwise die or suffer immensely as a result of their conditions. Over recent years, thousands of Canadians have benefited from receiving innovative therapies, often before their formal approval and without charge. Many Canadians with rare diseases can credibly say their lives were saved by the availability of a new treatment through a clinical study. But the new price controls are clearly already having an impact on clinical trials and research in Canada.

A quick search of the Health Canada Clinical Trials Database shows a startling decline in clinical trial approvals, looking at the most relevant time period, being the three months following the finalization of the *Patented Medicines Regulations*. The numbers of "no objection letters" issued by Health Canada was 222 in 2016, 217 in 2017, 258 in 2018, and dropped massively in 2019 to 153. No decline was experienced in the United States for the last four months of 2019 compared to previous periods. According to company representatives, their ability to compete internally for clinical trial sites in Canada has been increasingly compromised since the PMPRB put out the proposed regulatory changes starting in 2017.

The logic is irrefutable even if the PMPRB chooses not to believe it. Pharmaceutical companies will not invest in clinical studies in countries where they will not promptly launch new treatments and/or which do not encourage such investment by permitting prices that offer them a reasonable return on their drug development investments.

At present, Canada benefits from the disproportionately large number of clinical studies conducted here relative to its population and market size. The infrastructure and expertise that has allowed this to happen has been built carefully and steadily over the past 30 years but it is easily and significantly vulnerable to the impact of reduced research investments that will result from the proposed full PMPRB price regulation changes. CORD is very concerned that this impact has not been studied by the PMPRB – indeed, it has been dismissed entirely.

How else will the reduced corporate investment in clinical research harm Canadians? The health system infrastructure across Canada relies directly and indirectly on funding of clinical research. Many health institutions in general, research programs specifically, and individual expert scientists rely on corporate contributions and investments. As importantly, Canada has been able to leverage these investments to take part and even lead international collaborations; hence Canada's research reputation of being able to "punch above our weight."

The corollary to this is: when the research funding goes, so do the research and clinical experts. In recent years, Canada has fought back to stem the migration of Canadian trained clinicians and researchers. Our globally recognized research scientists are also expert clinicians, which translates into significant benefits for Canadian patients. Too much is at stake for the patient community to remain silent; we must raise the alarm before irrevocable harm occurs. We cannot accept the response that has been voiced by PMPRB leadership: "We will know in three to five years whether I [PMPRB] am right or you are right." As we know only too well, even the proposed changes, let along their implementation, have reduced investments in clinical research as well as introduction of vital medicines to Canada. While others experience the risks peripherally, patients and families experience them with their very lives and well-being.

Frankly, we cannot believe that the PMRPB and all those who are standing by their sides are ignorant of the information and implications we have presented here. Moreover, we cannot believe that the PMPRB and their supporters are uncaring of the impact on patients. So, what gives? is it really so important to make a political statement about the government trying to reduce the drug budget and, as their populous strategy, pressing down on drug prices?

Where Do We Go From Here?

As patients, we strongly endorse the balance of drug prices that are as low as possible to ensure as many patients as possible have access to the most appropriate medicine for their individual need AND prices that will give the developers sufficient return on investment to encourage further drug development. We believe this negotiation has to take place at local (national) levels within the context of global drug ecosystem. Drug prices should have international references but be customized (negotiated) to the priorities and capacities of local environments. We acknowledge there is no objective "right price" but these have to be negotiated within a consensus framework that assures fairness, consistency, and accountability.

As a patient association, we ask for the right to be part of the development of that consensus framework. It is not only ethically imperative but the only pathway to the most effective use of medicines and hence, the most cost-effective return on the health system investment.

Innovative medicines have been and will continue to be the most important form of treatment for patients with rare diseases. CORD fought hard for a Canadian Orphan Drug Regulatory Framework, which was developed but sadly never implemented. Nevertheless, the key elements of that framework have been enshrined and enacted in the revised Health Canada Regulatory Review of Drugs and Devices (R2D2), with a published Drugs for Rare Diseases Regulatory Pathway. We fought for and achieved changes to CADTH process for rare disease drugs (again without a separate pathway) that has improved the positive recommendations from about 62% to about 87% (albeit with criteria, including price reductions). Canadians with rare diseases will continue to advocate for equitable access to clinical trials, appropriate treatment, and monitoring and support to assure optimal utilization.

CORD is committed to working with all stakeholders to help advance a National Pharmacare program that will assure drug coverage for all, to help implement the P/T Expensive Drugs for Rare Diseases supplemental process to allow for timely access to rare disease drugs with managed access, and to design and implement a Canadian Rare Disease Drug Strategy that will assure Canadians will have the timely access to clinical trials and new therapies that provides optimal return for investment.

CORD is proud to lead Canadians with rare and non-rare diseases in the process to arrive at optimal guidelines for the implementation of pricing regulations. We cannot and will not give up our very important role of assuring full representation of patients through patient organization. For us, getting the PMPRB guidelines right is indeed the #FightforOurLives .

Sincerely,

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FDA versus Health Canada submissions (September 2018 – Present)

Drug Name	Active Ingredient	FDA Approval Date	Submitted to HC?	Additional Information
Tazverik	tazemetostat	23-Jan-20	No	To treat epithelioid sarcoma
Tepezza	teprotumumab-trbw	21-Jan-20	No	To treat Thyroid eye disease
Ayvakit	avapritinib	09-Jan-20	No	To treat adults with unresectable or metastatic gastrointestinal stromal tumor (GIST)
Ubrelvy	ubrogepant	23-Dec-19	No	to treat acute treatment of migraine with or without aura in adults
Enhertu	fam-trastuzumab deruxtecan-nxki	20-Dec-19	No	To treat metastatic breast cancer
Dayvigo	lemborexant	20-Dec-19	No	To treat insomnia
Caplyta	lumateperone tosylate	20-Dec-19	No	To treat schizophrenia
TissueBlue	Brilliant Blue G Ophthalmic Solution	20-Dec-19	No	Dye used in eye surgery
Padcev	enfortumab vedotin-ejfv	18-Dec-19	No	To treat refractory bladder cancer
Vyondys 53	golodirsen	12-Dec-19	No	To treat certain patients with Duchenne muscular dystrophy
Oxbryta	voxelotor	25-Nov-19	No	To treat sickle cell disease
Xcopri	cenobamate	21-Nov-19	No	To treat partial onset seizures
Givlaari	givosiran	20-Nov-19	No	To treat acute hepatic porphyria, a rare blood disorder
Adakveo	crizanlizumab-tmca	15-Nov-19	No	To treat patients with painful complication of sickle cell disease
Fetroja	cefiderocol	14-Nov-19	No	To treat patients with complicated urinary tract infections who have limited or no alternative treatment options
Brukinsa	zanubrutinib	14-Nov-19	no	To treat certain patients with mantle cell lymphoma, a form of blood cancer
Reblozyl	luspatercept–aamt	08-Nov-19	no	For the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell transfusions

ExEm Foam	air polymer-type A	07-Nov-19	no	A diagnostic agent used to assess fallopian tube patency (openness) in women with known or suspected infertility
Trikafta	elexacaftor/ivacaftor/tezacaftor	21-Oct-19	no	To treat patients 12 years of age and older with the most common gene mutation that causes cystic fibrosis
Reyvow	lasmiditan	11-Oct-19	no	For the acute treatment of migraine with or without aura, in adults
	fluorodopa F 18	10-Oct-19	no	A diagnostic agent for use in positron emission tomography (PET) to help diagnose adult patients with suspected Parkinsonian syndromes (PS)
Scenesse	afamelanotide	08-Oct-19	no	To increase pain-free light exposure in adult patients with a history of phototoxic reactions (damage to skin) from erythropoietic protoporphyria
Beovu	brolucizumab–dbll	07-Oct-19	yes	Treatment of wet age-related macular degeneration
Aklief	trifarotene	04-Oct-19	yes	For the topical treatment of acne vulgaris in patients 9 years of age and older
Ibsrela	tenapanor	12-Sep-19	yes	To treat irritable bowel syndrome with constipation in adults.
Nourianz	istradefylline	27-Aug-19	no	To treat adult patients with Parkinson's disease experiencing "off" episodes
Ga-68-DOTATOC	Ga-68-DOTATOC	21-Aug-19	no	For use with positron emission tomography (PET) for localization of somatostatin receptor positive neuroendocrine tumors (NETs)
Xenleta	lefamulin	19-Aug-19	no	To treat adults with community-acquired bacterial pneumonia
Rinvoq	upadacitinib	16-Aug-19	yes	To treat adults with moderately to severely active rheumatoid arthritis
Inrebic	fedratinib	16-Aug-19	yes	To treat adult patients with intermediate-2 or high-risk primary or secondary myelofibrosis
Rozlytrek	entrectinib	15-Aug-19	yes	To treat adult and pediatric patients 12 years of age and older with solid tumors
Wakix	pitolisant	14-Aug-19	no	To treat excessive daytime sleepiness (EDS) in adult patients with narcolepsy
	pretomanid	14-Aug-19	no	For treatment-resistant forms of tuberculosis that affects the lungs

Turalio	pexidartinib	02-Aug-19	no	To treat adult patients with symptomatic tenosynovial giant cell tumor
Nubeqa	darolutamide	30-Jul-19	yes	To treat adult patients with non-metastatic castration resistant prostate cancer
Accrufer	ferric maltol	25-Jul-19	no	To treat iron deficiency anemia in adults
Recarbrio	imipenem, cilastatin and relebactam	16-Jul-19	no	To treat complicated urinary tract and complicated intra-abdominal infections
Xpovio	selinexor	03-Jul-19	no	To treat adult patients with relapsed or refractory multiple myeloma (RRMM)
Vyleesi	bremelanotide	21-Jun-19	no	To treat hypoactive sexual desire disorder in premenopausal women.
Polivy	polatuzumab vedotin-piiq	10-Jun-19	yes	To treat adult patients with relapsed or refractory diffuse large B-cell lymphoma
Piqray	alpelisib	24-May-19	yes	To treat breast cancer
Vyndaqel	tafamidis meglumine	03-May-19	yes	To treat heart disease (cardiomyopathy) caused by transthyretin mediated amyloidosis (ATTR-CM) in adults
Skyrizi	risankizumab-rzaa	23-Apr-19	yes	To treat moderate-to-severe plaque psoriasis in adults who are candidates for systemic therapy or phototherapy
Balversa	erdafitinib	12-Apr-19	yes	To treat adult patients with locally advanced or metastatic bladder cancer
Evenity	romosozumab-aqqg	09-Apr-19	yes	To treat osteoporosis in postmenopausal women at high risk of fracture
Mayzent	siponimod	26-Mar-19	yes	To treat adults with relapsing forms of multiple sclerosis
Sunosi	solriamfetol	20-Mar-19	no	To treat excessive sleepiness in adult patients with narcolepsy or obstructive sleep apnea
Zulresso	brexanolone	19-Mar-19	no	To treat postpartum depression (PPD) in adult women
Egaten	triclabendazole	13-Feb-19	no	To treat fascioliasis, a parasitic infestation caused by two species of flatworms or trematodes that mainly the affect the liver, sometimes referred to as “liver flukes”
Cablivi	caplacizumab-yhdp	06-Feb-19	yes	To treat adult patients with acquired thrombotic thrombocytopenic purpura (aTTP)

Jeuveau	prabotulinumtoxinA-xvfs	01-Feb-19	yes	For the temporary improvement in the appearance of moderate to severe glabellar lines associated with corrugator and/or procerus muscle activity in adult patients
Ultomiris	ravulizumab	21-Dec-18	yes	To treat paroxysmal nocturnal hemoglobinuria (PNH)
Elzonris	tagraxofusp-erzs	21-Dec-18	no	To treat blastic plasmacytoid dendritic cell neoplasm (BPDCN)
Asparlas	calaspargase pegol-mknl	20-Dec-18	no	To treat acute lymphoblastic leukemia (ALL) in pediatric and young adult patients age 1 month to 21 years
Motegrity	prucalopride	14-Dec-18	no	To treat chronic idiopathic constipation
Xospata	gilteritinib	28-Nov-18	yes	To treat patients who have relapsed or refractory acute myeloid leukemia (AML)
Firdapse	amifampridine	28-Nov-28	no	To treat Lambert-Eaton myasthenic syndrome (LEMS) in adults
Vitrakvi	larotrectinib	26-Nov-18	yes	To treat patients whose cancers have a specific genetic feature (biomarker)
Daurismo	glasdegib	21-Nov-18	yes	To treat newly-diagnosed acute myeloid leukemia (AML) in adult patients
Gamifant	emapalumab-lzsgemapalumab-lzsg	20-Nov-18	no	To treat primary hemophagocytic lymphohistiocytosis (HLH)
Aemcolo	rifamycin	16-Nov-18	no	To treat travelers' diarrhea
Yupelri	revefenacin	09-Nov-18	no	To treat patients with chronic obstructive pulmonary disease (COPD)
Lorbrena	lorlatinib	02-Nov-18	yes	To treat patients with anaplastic lymphoma kinase (ALK)-positive metastatic non-small cell lung cancer
Xofluza	baloxavir marboxil	24-Oct-18	yes	To treat acute uncomplicated influenza in patients who have been symptomatic for no more than 48 hours.
Talzenna	talazoparib	16-Oct-18	yes	To treat locally advanced or metastatic breast cancer patients with a germline BRCA mutation.
Tegsedi	inotersen	05-Oct-18	yes	To treat polyneuropathy of hereditary transthyretin-mediated amyloidosis in adults
Revcovi	elapegademase-lvlr	05-Oct-18	no	To treat Adenosine Deaminase-Severe Combined Immunodeficiency (ADA-SCID)
Nuzyra	omadacycline	02-Oct-18	no	To treat community-acquired bacterial pneumonia and acute bacterial skin and skin structure infections

Seysara	sarecycline	01-Oct-18	no	To treat inflammatory lesions of non-nodular moderate to severe acne vulgaris in patients 9 years of age and older
Libtayo	cemiplimab-rwlc	28-Sep-18	yes	To treat cutaneous squamous cell carcinoma (CSCC)
Vizimpro	dacomitinib	27-Sep-18	yes	To treat metastatic non-small-cell lung cancer
Emgality	galcanezumab-gnlm	27-Sep-18	yes	For the preventive treatment of migraine in adults
Copiktra	duvelisib	24-Sep-18	no	To treat relapsed or refractory chronic lymphocytic leukemia, small lymphocytic lymphoma and follicular lymphoma
Ajovy	fremanezumab-vfrm	14-Sep-18	yes	For the preventive treatment of migraine in adults
Lumoxiti	moxetumomab pasudotox-tdfk	13-Sep-18	no	To treat hairy cell leukemia