

August 21, 2023

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

**Subject: PMPRB 2023 Interim Guidance**

Dear PMPRB Board Members,

The Canadian Organization for Rare Disorders (CORD) is the national alliance of over 100 rare disease patient organizations and representing approximately 3 million Canadians affected by rare diseases.

On behalf of the rare disease community, we would like to start our submission by thanking the PMPRB for the opportunity, once again, to provide input on PMPRB changes, this time on the PMPRB's proposed 2023 Interim Guidance. Since the 2017 introduction of broad-based proposed changes, CORD has participated in written submissions, face-to-face consultations, virtual meetings, PMPRB Modernization Steering Committee, PMPRB Technical Working Group, surveys, and submissions to the House Standing Committee on Health.

On the positive side, CORD appreciates PMPRB's openness to feedback and indeed modifications to proposed changes (somewhat) reflective of stakeholder input and other challenges. From a negative perspective, modifications based on feedback have been modest or superficial, often missing the significant potential for harm to the patient community when clinical trials do not include Canadians, when introduction of life-changing therapies are delayed, or wide disparities in industry and public payer perspectives on appropriate pricing further delay and deny patient access.

Not only has the PMPRB deviated from its original mandate to assure that Canadian prices were not excessive as an abuse of patent and in comparison to other countries, it has abandoned all pretext of assuring that Canadians have access to the best medicines. The so-called modernization reforms come at this precise time when innovative, advanced, life-altering, and highly effective medicines are being introduced for rare, complex and previously untreated conditions. While all countries struggle with creating space in their budgets for introduction of these therapies, often not explicitly acknowledging the lives and monies saved in the future, Canada has taken a uniquely draconian approach that is singularly focused on one objective, that is, driving prices as low as possible.

Even as these regulatory changes were being debated, the consequences were being experienced by Canadians and none more so than those with rare diseases, including slowdown or even withdrawal of clinical trials and relegation of Canada to second or lower tier, access.

Our concerns are not exaggerated fear-mongering. Following our previous submissions on the PMPRB reforms, we have updated and attached a list of medicines approved by the USFDA versus submissions to Canada's regulator, finding that since the first formal *Patented Medicines Regulations* changes were published on August 22, 2019, only 94 out of the 178 medicines already approved in the US have even been submitted for review by Health Canada as of August

15, 2023. This means that Canadians have access to only half of the medicines available just south of the border, including treatments for many cancers, Alzheimer's disease, diabetes, and other serious diseases.

For years now we have tried and failed to get through to the PMPRB. We were initially hopeful in 2018 when PMPRB set up a Steering Committee to advise on guidelines for the implementation of the proposed regulatory changes. Unfortunately, we all learned that the guidelines were already written and there was little interest in receiving advice and even less interest in any meaningful change to the guidelines. The so-called "Steering Committee" didn't steer anything – we were there to give the impression that the PMPRB is engaging stakeholders, when in fact all of our input was completely disregarded.

This pattern of selective listening and response has continued throughout the entire PMPRB "reform" process, with tightly controlled forums, no open dialogue between staff/board and stakeholders, and no opportunity to deliberate on alternative approaches to pricing. The PMPRB promised to make "significant changes" to its initial proposed implementation plan in light of significant stakeholder concerns about the impacts of the new pricing system on patient access to needed medicines; however, subsequent guidelines offered minimal changes.

Perhaps the most concerning aspect of the entire PMPRB reform was the revelation in 2021 that the board had developed a communications plan and engaged in advocacy against all who have expressed genuine concerns about the reforms. This is an egregious breach of the PMPRB's duty of neutrality as a quasi-judicial government agency.

**In this context, and with respect to the PMPRB's approach for the interim and final guidelines, CORD urges the PMPRB to engage in more meaningful consultations with stakeholders and explore the impact of its pricing policies on patients.**

This will only be possible if the PMPRB reviews how the changes will operate in practice and with case studies, which was a key recommendation from CORD representatives throughout earlier guidelines modernization efforts. The PMPRB should also act less like a punitive body and more like a "public good" agency. It should collaborate with patients, clinicians, payers, and other stakeholders to arrive at pricing guidelines that work for everyone.

Perhaps most importantly from CORD's perspective, the PMPRB should also ensure that any approach taken enables and supports federal and provincial rare disease strategies that are critical for our community.

Thank you for the opportunity to provide input.

Sincerely,



Durhane Wong-Rieger, PhD  
President & CEO  
durhane@raredisorders.ca

Encl.

Medicines that have been approved by the FDA and/or submitted to Health Canada since August 22, 2019 - updated to August 15, 2023					
Drug Name	Active Ingredient	FDA Approval Date	Submitted to HC?	HC Submission/Approval Notes	Treatment / Use
Elrexfio	elranatamab-bcmm	14-Aug-23	Yes	New active substance; Being reviewed under the Notice of Compliance with Conditions Guidance	To treat adults with relapsed or refractory multiple myeloma who have received at least four prior lines of therapy
Ilalvey	talquetamab-tgvs	09-Aug-23	No		To treat adults with relapsed or refractory multiple myeloma who have received at least four prior therapies
Ilervay	avacincaptad pegol	04-Aug-23	No		To treat geographic atrophy secondary to age-related macular degeneration
Zurzuvae	zuranolone	04-Aug-23	No		To treat postpartum depression
Xdemvy	lotilaner	25-Jul-23	No		To treat Demodex blepharitis
Vanflyta	quizartinib	20-Jul-23	No		To use as part of a treatment regimen for newly diagnosed acute myeloid leukemia that meets certain criteria
Beyfortus	nirsevimab-alip		Yes	Issued NOC in Apr. 2023	To prevent respiratory syncytial virus (RSV) lower respiratory tract disease
Ngenla	somatrogon-ghla	27-Jun-23	Yes	Issued NOC in Oct. 2021	To treat growth failure due to inadequate secretion of endogenous growth hormone
Rystiggo	rozanolixumab-noli	26-Jun-23	No		To treat generalized myasthenia gravis in adults who are anti-acetylcholine receptor- or anti-muscle-specific tyrosine kinase antibody-positive
Itfulo	ritlecitinib	23-Jun-23	Yes	New active substance, Feb. 2023	To treat severely patchy hair loss
Ilolumvi	glofitamab-gxbm	15-Jun-23	Yes	Issued NOC in Mar. 2023	To treat diffuse large B-cell lymphoma, not otherwise specified, or large B-cell lymphoma arising from follicular lymphoma after two or more lines of systemic therapy
Inpefa	sotagliflozin	26-May-23	No		To treat heart failure
Ilaxlovid	nirmatrelvir, ritonavir	25-May-23	Yes	Issued NOC in Jan. 2022	To treat mild-to-moderate COVID-19 in adults at high risk for progression to severe COVID-19
Xacduro	sublactam, durlobactam	23-May-23	No		To treat hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia caused by susceptible isolates of Acinetobacter baumannii-calcoaceticus complex
Epkinly	epcoritamab-bysp	19-May-23	Yes	New active substance; Being reviewed under the Notice of Compliance with Conditions Guidance	To treat relapsed or refractory diffuse large B-cell lymphoma (not otherwise specified) and high-grade B-cell lymphoma after two or more lines of systemic therapy
Veozah	fezolinetant	12-May-23	No		To treat moderate to severe hot flashes caused by menopause
Elfabrio	pegunigalsidase alfa-ixwj	09-May-23	No		To treat confirmed Fabry disease
Qalsody	tofersen	25-Apr-23	No		To treat amyotrophic lateral sclerosis in adults who have a SOD1 gene mutation
Joenja	leniolisib	24-Mar-23	No		To treat activated phosphoinositide 3-kinase delta syndrome
Rezzayo	rezafungin	22-Mar-23	No		To treat candidemia and invasive candidiasis
Ilzynz	retifanlimab-dlwr	22-Mar-23	No		To treat metastatic or recurrent locally advanced Merkel cell carcinoma
Daybue	trofinetide	10-Mar-23	No		To treat Rett syndrome
Zavzpret	zavegepant	09-Mar-23	No		To treat migraine
Skyclarys	omaveloxolone	28-Feb-23	No		To treat Friedrich's ataxia
Elispari	sparsentan	17-Feb-23	No		To reduce proteinuria in adults with primary immunoglobulin A nephropathy at risk of rapid disease progression
Lamzede	velmanase alfa-tycv	16-Feb-23	No		To treat non-central nervous system manifestations of alpha-mannosidosis
Jesduvroq®	daprodustat	01-Feb-23	Yes	Part of 'aligned review' with a health technology assessment organization	To treat anemia caused by chronic kidney disease for adults on dialysis for at least four months
Orserdu	elacestrant	27-Jan-23	No		To treat estrogen receptor-positive, human epidermal growth factor receptor 2-negative, ESR1-mutated, advanced or metastatic breast cancer with disease progression following at least one line of endocrine therapy
Ilbypirca	pirtobrutinib	27-Jan-23	No		To treat relapsed or refractory mantle cell lymphoma in adults who have had at least two lines of systemic therapy, including a BTK inhibitor
Ilrenzavvy	bexagliflozin		No		To improve glycemic control in adults with type 2 diabetes mellitus as an adjunct to diet and exercise
Ilqembi	lecanemab-irmb	06-Jan-23	Yes	New active substance	To treat Alzheimer's disease
Ilriumvi	ublituximab-xiiy	28-Dec-22	No		To treat relapsing forms of multiple sclerosis



Lunsumio	mosunetuzumab-axgb	22-Dec-22	No		To treat adults with relapsed or refractory follicular lymphoma, a type of non-Hodgkin lymphoma
Sunlenca	lenacapavir	12-Dec-22	Yes	NOC in Dec. 2022	To treat adults with HIV whose HIV infections cannot be successfully treated with other available treatments due to resistance, intolerance, or safety considerations
Krazati	adagrasib	12-Dec-22	No		To treat KRAS G12C-mutated locally advanced or metastatic non-small cell lung cancer in adults who have received at least one prior systemic therapy
Bezlidhia	fluticasone propionate	01-Dec-22	No		To treat adults with relapsed or refractory acute myeloid leukemia with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation
Uzielid	insulin glargine	18-Nov-22	No		To delay the onset of stage 3 type 1 diabetes
Elahere	mirvetuximab soravtansine-gynx	14-Nov-22	No		To treat patients with recurrent ovarian cancer that is resistant to platinum therapy
Tecvayli	teclistamab-cqyv	25-Oct-22	Yes	Being reviewed under the Notice of Compliance with Conditions Guidance	To treat relapsed or refractory multiple myeloma among adults who have received at least four specific lines of therapy
Imjudo	tremelimumab	21-Oct-22	Yes	Submitted May 2022	To treat unresectable hepatocellular carcinoma
Eytgobi	futibatinib	30-Sep-22	No		To treat intrahepatic cholangiocarcinoma harboring fibroblast growth factor receptor 2 (FGFR2) gene fusions or other
Relyvrio	sodium phenylbutyrate/tauroursod	29-Sep-22	Yes	NOC in June 2022	To treat amyotrophic lateral sclerosis (ALS)
Terlivaz	terlipressin	14-Sep-22	No		To improve kidney function in adults with hepatorenal syndrome with rapid reduction in kidney function
Rolvedon	eflapegrastim	09-Sep-22	No		To decrease the incidence of infection in patients with non-myeloid malignancies receiving myelosuppressive anti-cancer drugs associated with clinically significant incidence of febrile neutropenia
Sotyktu	deucravacitinib	09-Sep-22	Yes	New active substance	To treat moderate-to-severe plaque psoriasis
Daxxify	daxibotulinumtoxinA-ianm	07-Sep-22	No		To treat moderate-to-severe glabellar lines associated with corrugator and/or procerus muscle activity
Spevigo	spesolimab-sbzo	01-Sep-22	Yes	Being reviewed under the Priority Review Policy	To treat generalized pustular psoriasis flares
Xenpozyme	Olipudase alfa	31-Aug-22	Yes	Being reviewed under the Priority Review Policy	To treat Acid Sphingomyelinase Deficiency
Amvuttra	vutrisiran	13-Jun-22	Yes	New active substance	To treat polyneuropathy of hereditary transthyretin-mediated amyloidosis
Vtama	tapinarof	23-May-22	Yes	New active substance	To treat plaque psoriasis
Mounjaro	tirzepatide	13-May-22	Yes	Submitted February 2022	To improve blood sugar control in diabetes
Voquezna	vonoprazan, amoxicillin, and clarithromycin	03-May-22	No		To treat Helicobacter pylori infection
Camzyos	mavacamten	28-Apr-22	Yes	Submitted January 2022	To treat certain classes of obstructive hypertrophic cardiomyopathy
Qivjoo	oteseconazole	26-Apr-22	No		To reduce the incidence of recurrent vulvovaginal candidiasis (RVVC) in females with a history of RVVC who are not of reproductive potential
Pluvicto	lutetium (177Lu) vipivotide tetraxetan	23-Mar-22	Yes	Issued NOC in August 2022	To treat prostate-specific membrane antigen-positive metastatic castration-resistant prostate cancer following other therapies
Opdualag	nivolumab and relatimab-rmbw	18-Mar-22	Yes	New active substance	To treat unresectable or metastatic melanoma
Ztalmy	ganaxolone	18-Mar-22	No		To treat seizures in cyclin-dependent kinase-like 5 deficiency disorder
Donjo	pacritinib	28-Feb-22	No		To treat intermediate or high-risk primary or secondary myelofibrosis in adults with low platelets
Byrukynd	mitapivat	17-Feb-22	No		To treat hemolytic anemia in pyruvate kinase deficiency
Enjaymo	rituximab-jome	04-Feb-22	No		To decrease the need for red blood cell transfusion due to hemolysis in cold agglutinin disease
Abysmo	faricimab-svoa	28-Jan-22	Yes	NOC May 2022	To treat neovascular (wet) aged-related macular degeneration and diabetic macular edema



<b>Kimtrak</b>	tebentafusp-tebn	25-Jan-22	Yes	Issued NOC June 2022	To treat unresectable or metastatic uveal melanoma
<b>Cibinqo</b>	abrocitinib	14-Jan-22	Yes	New active substance Part of 'aligned review' with a health technology assessment organization	To treat refractory, moderate-to-severe atopic dermatitis
<b>Quviviq</b>	daridorexant	07-Jan-22	Yes	Submitted October 2021	To treat insomnia
<b>Adbry</b>	efalokinumab-ldrm	27-Dec-21	Yes	Issued NOC in October 2021	To treat moderate-to-severe atopic dermatitis
<b>Beqvio</b>	inclisiran	22-Dec-21	Yes	NOC July 2021	hypercholesterolemia or clinical atherosclerotic cardiovascular disease as an
<b>Vyvgart</b>	efgartigimod alfa-fcab	17-Dec-21	Yes	New active substance	To treat generalized myasthenia gravis
<b>Tezspire</b>	tezepelumab-ekko	17-Dec-21	Yes	Submitted October 2021	To treat severe asthma as an add-on maintenance therapy
<b>Livtency</b>	maribavir	23-Nov-21	Yes	Submitted March 2022	To treat post-transplant cytomegalovirus (CMV) infection/disease that does not respond (with or without genetic mutations that cause resistance) to available antiviral treatment for CMV
<b>Voxzogo</b>	vosoritide	19-Nov-21	No		To improve growth in children five years of age and older with achondroplasia and open epiphyses
<b>Besremi</b>	ropeginterferon alfa-2b-njft	12-Nov-21	No		To treat polycythemia vera, a blood disease that causes the overproduction of red blood cells
<b>Scemblix</b>	asciminib	29-Oct-21	Yes	Submitted August 2021	To treat Philadelphia chromosome-positive chronic myeloid leukemia with disease that meets certain criteria
<b>Tavneos</b>	avacopan	07-Oct	Yes	Submitted May 2021	To treat severe acute anti-neutrophil cytoplasmic autoantibody-associated vasculitis (granulomatosis with polyangiitis)
<b>Elvmarli</b>	maralixibat	29-Sep-21	Yes	Priority Review Jan. 2023	To treat cholestatic pruritus associated with Alagille syndrome
<b>Qulipta</b>	atogepant	28-Sep-21	Yes	Submitted July 2021	To prevent episodic migraines
<b>Invdak</b>	lisotumab vedotin-tftv	20-Sep-21	No		To treat recurrent or metastatic cervical cancer with disease progression on or after chemotherapy
<b>Exkivity</b>	mobocertinib	15-Sep-21	No		To treat locally advanced or metastatic non-small cell lung cancer with epidermal growth factor receptor exon 20 insertion mutations
<b>Skytrofa</b>	teriparatid-tcgd	25-Aug-21	No		To treat short stature due to inadequate secretion of endogenous growth hormone
<b>Korsuva</b>	difelikefalin	23-Aug-21	Yes	Submitted October 2021	To treat moderate-to-severe pruritus associated with chronic kidney disease in certain populations
<b>Belireg</b>	belzutifan	13-Aug-21	Yes	Submitted September 2021.	To treat von Hippel-Lindau disease under certain conditions
<b>Nexviazyme</b>	avalglucosidase alfa-ngpt	06-Aug-21	Yes	Submitted November 2020	To treat late-onset Pompe disease
<b>Saphnelo</b>	anifrolumab-fria	30-Jul-21	Yes	Submitted December 2020	To treat moderate-to-severe systemic lupus erythematosus along with standard therapy
<b>Bylvay</b>	odevixibat	20-Jul-21	Yes	Being reviewed under the Priority Review Policy	To treat pruritus
<b>Bezurock</b>	belumosudil	16-Jul-21	Yes	Submitted January 2021. Being reviewed under the Priority Review Policy	To treat chronic graft-versus-host disease after failure of at least two prior lines of systemic therapy
<b>fexinidazole</b>	fexinidazole	16-Jul-21	No		To treat human African trypanosomiasis caused by the parasite <i>Trypanosoma brucei gambiense</i>
<b>Merendia</b>	finerenone	09-Jul-21	Yes	Submitted April 2021	To reduce the risk of kidney and heart complications in chronic kidney disease associated with type 2 diabetes
<b>Bylaze</b>	asparaginase erwinia chrysanthemii (recombinant)-rywn	30-Jun-21	Yes	Submitted September 2021.	To treat acute lymphoblastic leukemia and lymphoblastic lymphoma in patients who are allergic to E. coli-derived asparaginase products, as a component of a chemotherapy regimen
<b>Aduhelm</b>	aducanumab-avwa	07-Jun-21	Yes	Submitted June 2021	To treat Alzheimer's disease
<b>Brexafemme</b>	ibrexafungerp	01-Jun-21	No		To treat vulvovaginal candidiasis
<b>Lybalvi</b>	olanzapine and samidorphan	28-May-21	No		To treat schizophrenia and certain aspects of bipolar I disorder
<b>Truseqiq</b>	infigratinib	28-May-21	Yes	Being reviewed under the Notice of Compliance with Conditions Guidance	To treat cholangiocarcinoma whose disease meets certain criteria
<b>Lumakras</b>	sotorasib	28-May-21	Yes	Being reviewed under the Notice of Compliance with Conditions Guidance	To treat types of non-small cell lung cancer
<b>Rybrevant</b>	amivantamab-vmjw	21-May-21	Yes	Submitted July 2021	To treat a subset of non-small cell lung cancer



Empaveli	pegcetacoplan	14-May-21	Yes	Submitted Mar. 2023	To treat paroxysmal nocturnal hemoglobinuria
Zynlonta	Encastuximab tesirine-lyyl	23-Apr-21	No		To treat certain types of relapsed or refractory large B-cell lymphoma
Jemperli	dostarlimab-gxly	22-Apr-21	Yes	Submitted April 2021. Being reviewed under the Notice of Compliance with Conditions Guidance	To treat endometrial cancer
Nextstellis	drospirenone and estetrol tablets	15-Apr-21	Yes	NOC March 2021	To prevent pregnancy
Belbree	viloxazine	02-Apr-21	No		For the treatment of attention deficit hyperactivity disorder
Begalogue	dasiglucagon	22-Mar-21	No		To treat severe hypoglycemia
Bonvory	ponesimod	18-Mar-21	Yes	Submitted June 2020	To treat patients with relapsing forms of multiple sclerosis
Botivda	tivozanib	10-Mar-21	No		To treat patients with renal cell carcinoma
Azstarys	serdexmethylphenidate and dexmethylphenidate	02-Mar-21	No		For the treatment of Attention Deficit Hyperactivity
Pepaxto	melphalan flufenamide	26-Feb-21	No		For the treatment of certain patients with relapsed or refractory multiple myeloma
Nulibry	fosdenopterin	26-Feb-21	No		To treat patients with the rare genetic disease molybdenum cofactor deficiency Type A
Amondys 45	casimersen	25-Feb-21	No		For the treatment of Duchenne muscular dystrophy
Cosela	trilaciclib	12-Feb-21	No		To mitigate chemotherapy-induced myelosuppression in adult patients with small cell lung cancer
Evkeeza	evinacumab-dgnb	11-Feb-21	No		For the treatment of homozygous familial hypercholesterolemia
Ukoniq	umbralisib	05-Feb-21	No		For the treatment of certain patients with marginal zone lymphoma and follicular lymphoma
Eepmetko	tepotinib	03-Feb-21	Yes	Submitted December 2020	To treat non-small cell lung cancer
Eupkynis	voclosporin	22-Jan-21	No		To treat lupus nephritis
Eabenuva	cabotegravir and rilpivirine	21-Jan-21	Yes	Issued NOC in April 2020	To treat HIV
Verquvo	vericiguat	19-Jan-21	Yes	Submitted January 2021	To treat chronic heart failure
Gemtesa	vibegron	23-Dec-20	No		To treat overactive bladder
Ebanga	ansuvimab-zykl	21-Dec-20	No		To treat Ebola
Orgovyx	relugolix	18-Dec-20	Yes	New active substance	To treat advanced prostate cancer
Margenza	margetuximab (anti-HER2 mAb)	16-Dec-20	No		To treat HER2+ breast cancer
Klisyri	tirbanibulin	14-Dec-20	Yes	Submitted July 2022	To treat actinic Keratosis of the face or scalp
Orladeyo	berotralstat	03-Dec-20	Yes	Submitted August 2021	To treat patients with hereditary angioedema
Danyelza	naxitamab-ggqk	25-Nov-20	No		To treat high-risk refractory or relapsed neuroblastoma
Encivree	setmelanotide	25-Nov-20	Yes	New active substance	To treat obesity and the control of hunger associated with pro-opiomelanocortin deficiency, a rare disorder that causes severe obesity that begins at an early age
Exlumo	lumasiran	23-Nov-20	Yes	Submitted August 2021	To treat hyperoxaluria type 1
Exkinvy	lonafarnib	20-Nov-20	No		To treat rare conditions related to premature aging
Exklury	remdesivir	22-Oct-20	Yes	NOC July 2020	FDA: To treat COVID-19 Antivirals for systemic use HC:
Exmazeb	atoltivimab, maftivimab, and odesivimab-ebgn	14-Oct-20	No		To treat ebola virus
Gavreto	pralsetinib	04-Sep-20	Yes	Submitted November 2020	To treat non-small lung cancer
Enspryng	satralizumab-mwge	14-Aug-20	Yes	Issued NOC in June 2020	To treat neuromyelitis optica spectrum disorder
Viltepso	viltolarsen	12-Aug-20	No		To treat Duchenne muscular dystrophy
Olinvyk	oliceridine	07-Aug-20	No		To manage acute pain in certain adults
Evrysdi	risdiplam	07-Aug-20	Yes	Submitted Oct. 2020, under review	To treat spinal muscular atrophy
Lampit	nifurtimox	06-Aug-20	No		To treat Chagas disease in certain pediatric patients younger than age 18
Bienrep	belantamab mafodotin-blmf	05-Aug-20	No		To treat multiple myeloma
Monjuvi	tafasitamab-cxix	31-Jul-20	Yes	Submitted December 2020	To treat relapsed or refractory diffuse large B-cell lymphoma
Inqovi	decitabine and cedazuridine	07-Jun-20	Yes	NOC July 2020	To treat adult patients with myelodysplastic syndromes
Bukobia	fostemsavir	02-Jun-20	Yes	Submitted April 2021	To treat HIV
Byfavo	remimazolam	02-Jun-20	No		For sedation
Bojolvi	triheptanoin	30-Jun-20	Yes	Submitted August 2020	To treat molecularly long-chain fatty acid oxidation disorders
Zepzelca	lurbinectedin	15-Jun-20	Yes	Submitted January 2021	To treat metastatic small cell lung cancer
Uplizna	inebilizumab-cdon	11-Jun-20	No		To treat neuromyelitis optica spectrum disorder
Artesunate	artesunate	26-May-20	No		To treat severe malaria



Qinlock	ripretinib	15-May-20	Yes	Submitted March 2020, NOC June 2020	To treat advanced gastrointestinal-stromal tumors
Retevmo	seliperatinib	08-May-20	Yes	Submitted Oct. 2020, being reviewed under NoC with conditions guidance	To treat lung and thyroid cancers
Tabrecta	capmatinib	06-May-20	Yes	Submitted September 2021.	To treat patients with non small cell lung cancer
Ongentys	opicapone	24-Apr-20	No		To treat patients with Parkinson's disease experiencing "off" episodes
Trodelvy	sacituzumab govitecan-hziy	22-Apr-20	Yes	Submitted March 2021	To treat adult patients with metastatic triple-negative breast cancer who received at least two prior therapies for metastatic disease
Pemazyre	pemigatinib	17-Apr-20	Yes	Submitted Oct. 2020	To treat certain patients with cholangiocarcinoma, a rare form of cancer that forms in bile ducts
Tukysa	tucatinib	17-Apr-20	yes	NDS February 2020 (priority review), N	Advanced unresectable or metastatic HER2-positive breast cancer
Koselugo	selumetinib	10-Apr-20	Yes	Submitted November 2020	Neurofibromatosis type 1, a genetic disorder of the nervous system causing tumors to grow on nerves
Zeposia	ozanimod	25-Mar-20	yes	NDS in December 2019	Relapsing forms of multiple sclerosis
Isturisa	osilodrostat	06-Mar-20	Yes	New active substance	Cushing's disease
Sarclisa	isatuximab-irfc	03-Mar-20	yes	NDS July 2019, NOC April 2020	Multiple Myeloma
Nurtec ODT	rimegepant	27-Feb-20	Yes	New active substance	Migraine
Barhemsys	amisulpride	26-Feb-20	Yes	Cancelled by sponsor in 2021	Nausea and vomiting
Vyepti	eptinezumab-jjmr	21-Feb-20	Yes	Submitted March 2020	Migraine
Nexletol	bempedoic acid	21-Feb-20	No		Heterozygous familial hypercholesterolemia or established atherosclerotic CV disease
Pizensy	lactitol	12-Feb-20	No		Chronic idiopathic constipation (CIC) in adults
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	Yes	Submitted October 2021	to treat acute treatment of migraine with or without aura in adults
Enhertu	fam-trastuzumab deruxtecan-nxki	20-Dec-19	yes	NDS (2017/18) and SNDS (Sept 2019)	To treat metastatic breast cancer
Dayvigo	lemborexant	20-Dec-19	yes	NDS January 2020	To treat insomnia
Caplyta	lumateperone tosylate	20-Dec-19	No		To treat schizophrenia
Padcev	enfortumab vedotin-efjv	18-Dec-19	Yes	Submitted May 2021	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	Yes	Submitted July 2022	To treat partial onset seizures
Givlaari	givosiran	20-Nov-19	Yes	Submitted April 2020. NOC October 2020	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	Yes	Submitted September 2020. NOC March 2021	To treat certain patients with mantle cell lymphoma, a form of blood cancer
Reblozyl	luspatercept-aamt	08-Nov-19	Yes	Not Applicable as of April 2020	For the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell transfusions
Trikafta	elexacaftor/ivacaftor/tezacaftor	21-Oct-19	Yes	Submitted December 2020. NOC June 2021	To treat patients 12 years of age and older with the most common gene mutation that causes cystic fibrosis
Reyvow	lasmiditan	11-Oct-19	Yes	Submitted April 2020. Cancelled by sponsor	For the acute treatment of migraine with or without aura, in adults
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	brolocuzumab-dbll	07-Oct-19	yes	submitted May 2019; approved March 2020	Treatment of wet age-related macular degeneration
Aklief	trifarotene	04-Oct-19	yes	submitted January 2019, approved November 2019	For the topical treatment of acne vulgaris in patients 9 years of age and older
Ibsrela	tenapanor	12-Sep-19	yes	submitted June 2019, approved April 2020	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
<b>TOTAL:</b>				<b>94 HC/178 FDA</b>	
Sources: <a href="https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023">https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023</a>					
<a href="https://www.canada.ca/en/health-canada/services/drug-health-product-review-approval/submissions-under-review.html">https://www.canada.ca/en/health-canada/services/drug-health-product-review-approval/submissions-under-review.html</a>					
Note: Due to the pandemic, there were some delays in FDA Plant Inspections and product approvals.					
See: <a href="https://www.bloomberg.com/news/articles/2021-05-05/pandemic-delayed-some-fda-plant-inspections-product-">https://www.bloomberg.com/news/articles/2021-05-05/pandemic-delayed-some-fda-plant-inspections-product-</a>					