



December 20, 2023

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

Subject: PMPRB Scoping Paper consultation (2023)

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 our patients and families, I would like to commend you for taking a refreshed perspective on the guidelines development process. We are especially heartened that the PMPRB appears to be returning to its core mandate, which hopefully will help turn the page on what has been a very traumatic chapter for CORD and the rare disease community.

Based on the media hype and disinformation campaigns, one would be excused in imagining that the hospitals and pharmacies are overflowing with expensive rare disease therapies. Nothing could be further from the truth, with less than 5% of rare diseases having an approved treatment and many of these delayed in their entry to Canada, and some, not at all.

However, our community wants to move forward constructively and not dwell on the collective stress and apprehension caused by the proposed changes over the past seven years. On behalf of Canadian rare disease families, of which my family is one, I urge you to keep us forefront in your minds as you deliberate on your new guidelines. Here are examples of why appropriate pricing regulations are essential to rare disease families.

- A young woman diagnosed with fibrodysplasia ossificans progressiva (FOP), a rare musculoskeletal condition whereby, after birth and progressively through life, muscles and tendons are gradually transformed into bone (similar to the condition affecting Celine Dion). A treatment developed in Quebec more than eight years ago has now been approved by Health Canada and recommended by CADTH. It is awaiting consideration for pricing negotiations but the process could be further delayed by the fact that drug was just approved by the FDA this past August so there are limited international benchmarks.
- A 10-year-old boy with Neurofibromatosis type 1 (NF1), has undergone numerous surgeries to remove tumours throughout his body, and now has new growths near his spine. An oral therapy that has been shown to shrink the tumours, thus reducing pain and avoiding surgery, was approved in 2020 and 2021 in the USA and EU, respectively. Submission to Health Canada was delayed several years until pricing and reimbursement had been secured elsewhere. Several children could have avoided surgery with more timely access.
- A 21-year-old student with homozygous familial hypercholesterolemia (HOFH), a genetic condition characterized by very high LDL-C levels, travels four hours every two weeks to

receive plasma transfusions. However, these are not very effective and she has experienced several cardiovascular events. A new drug infused monthly is effective in reducing LDL-C by nearly 50%. Its submission in Canada was delayed for about two years until after EU and USA had negotiated pricing and reimbursement. She suffered her last attack during that delay.

These are only a few examples of the many serious impacts on rare disease patients, with many companies choosing to delay or even forego bringing life-altering and life-saving therapies to Canada, citing the unreasonable, un-negotiable and unprecedented changes in how PMPRB operates, which would have put our pricing ceilings far below other OECD countries. Rare disease patients are still suffering from delayed and no entry of therapies during these past years, many of which are the first for that condition.

To illustrate these challenges, we have updated and attached a list of medicines approved by the United States Food & Drug Agency versus submissions to Health Canada, finding that since the first formal *Patented Medicines Regulations* changes were published on August 22, 2019, only 102 out of the 197 medicines already approved in the US have even been submitted for review by Health Canada as of December 6, 2023. This means that Canadians have access to only about half of the medicines available just south of the border, including treatments for many cancers, Alzheimer's disease, diabetes, and other serious diseases. These are deliberate choices made by companies driven in part by the uncertainty surrounding compliant pricing and the potential impacts on pricing in other markets.

As patients, we wholeheartedly endorse the essence of the PMPRB's mandate, which is to prevent excessive prices for patented medicines and report on pricing and research trends in Canada. Sadly, the previous reforms strayed far from this objective. The focus on driving prices down without adequately considering the impact on patient access contradicts the foundation upon which this organization was built.

In light of these issues, CORD takes some comfort that the PMPRB has begun to engage in more meaningful consultations, and this starts with considering the real impact of pricing policies on patients. This can only be achieved through a thorough review of operational changes with practical case studies. Collaboration with patients, clinicians, payers, and stakeholders is crucial in formulating pricing guidelines that serve everyone's interests.

A measure of success or failure for the PMPRB needs to be an understanding of how its regulatory role – reviewing prices for potential excessiveness – is either enabling or obstructing the opportunity to access rare disease research and medicines in Canada.

As the PMPRB begins to reflect on its guidelines approach, we encourage you to consider the following key questions:

1. How will the PMPRB changes enable the implementation of Canada's rare disease drug strategy?
2. How would updated price ceilings affect the capacity for health systems and payers to negotiate dynamic agreements that provide access to medicines and the full range of care required for precision medicines, including cell and gene therapies? In Canada, our payers are getting creative and negotiating managed access programs and outcomes-based agreements. How can the PMPRB support these efforts, which are the hallmark of access for rare diseases in other countries and, increasingly, in Canada?

3. How do you plan to benchmark or monitor for excessive prices when a medicine is only available in a jurisdiction that isn't within the PMPRB11, for example, the US, where most medicines are made available first?

CORD has been active in the development and implementation of rare disease strategies and we are ready to bring our collective ingenuity to bear on these and other questions as you move forward. We would welcome the opportunity to take part in any guidelines steering groups, as we have done in the past.

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 December 6, 2023					
Drug Name	Active Ingredient	FDA Approval Date	Submitted to HC?	HC Submission/Approval Notes	Treatment / Use
Ogiveo	nirogacestat	27-Nov-23	No		To treat adults with progressing desmoid tumors who require systemic treatment
Trugap	capiwasertib	16-Nov-23	Yes	New active substance	To treat breast cancer that meets certain disease criteria
Ryzneuta	efbemalenograstim alfa-vuxw	16-Nov-23	No		To treat neutropenia
Auqtyro	repotrectinib	15-Nov-23	No		To treat ROS1-positive non-small cell lung cancer
Defencath	taurolidine, heparin	15-Nov-23	No		To reduce the incidence of catheter-related bloodstream infections in adults with kidney failure receiving chronic hemodialysis through a central venous catheter
Fruzaqla	fruquintinib	08-Nov-23	No		To treat refractory, metastatic colorectal cancer
Loqtorzi	toripalimab-tpzi	27-Oct-23	No		To treat recurrent or metastatic nasopharyngeal carcinoma when used together with or following other therapies
OmvoH	mirikizumab-mrkz	26-Oct-23	No		To treat ulcerative colitis
Amgamree	vamorolone	26-Oct-23	No		To treat Duchenne muscular dystrophy
Bimzelx	bimekizumab		Yes	Issued NOC	To treat moderate to severe plaque psoriasis in adults who are candidates for systemic therapy or phototherapy
Elbrysq	zilucoplan	17-Oct-23	Yes	New active substance	To treat generalized myasthenia gravis in adults who are anti-acetylcholine receptor (AChR) antibody positive
Melsipity	etrasimod	12-Oct-23	Yes	New active substance	To treat moderately to severely active ulcerative colitis in adults
Rivfloza	nedosiran	29-Sep-23	No		To lower urinary oxalate levels in patients 9 years and older with primary hyperoxaluria type 1 and relatively preserved kidney function
Pombiliti	cipaglucosidase alfa-atga	28-Sep-23	No		To treat late-onset Pompe disease
Exxua	gepirone	22-Sep-23	No		To treat major depressive disorder
Oijaara	mometinib	15-Sep-23	No		To treat intermediate or high-risk myelofibrosis in adults with anemia
Aphexda	motixafortide	08-Sep-23	No		To use with filgrastim (G-CSF) to mobilize hematopoietic stem cells to the peripheral blood for collection and subsequent autologous transplantation in patients with multiple myeloma
Neopoz	pezelimab-bbfg	18-Aug-23	No		To treat patients 1 year old and older with CD55-deficient protein-losing enteropathy (PLE), also known as CHAPLE disease
Sohonos	palovarotene	16-Aug-23	Yes	Issued NOC in Jan. 2022	To reduce the volume of new heterotopic ossification in adults and pediatric patients (aged 8 years and older for females and 10 years and older for males) with fibrodysplasia ossificans progressiva
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
Elvelve	talquetamab-tgvs	09-Aug-23	Yes	New active substance	To treat adults with relapsed or refractory multiple myeloma who have received at least four prior therapies
Elervay	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
Ngenia	somatrogon-ghia	27-Jun-23	Yes	Issued NOC in Oct. 2021	To treat growth failure due to inadequate secretion of endogenous growth hormone
Rystiggo	rozanolixizumab-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
Elitfulo	ritlicitinib	23-Jun-23	Yes	New active substance, Feb. 2023	To treat severely patchy hair loss
Bolumvi	glofitamab-gxhm	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
Elaxlovid	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	sulbactam, 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
Elpkinly	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-iwvj	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	Yes	New active substance	To treat activated phosphoinositide 3-kinase delta syndrome
Rezzayo	rezafungin	22-Mar-23	No		To treat candidemia and invasive candidiasis
Bynzy	retifanlimab-diwr	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
Elspari	sparsentan	17-Feb-23	No		To reduce proteinuria in adults with primary immunoglobulin A nephropathy at risk of rapid disease progression
Lamzedo	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
Bypirca	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
Brenzavvy	bexagliflozin		No		To improve glycemic control in adults with type 2 diabetes mellitus as an adjunct to diet and exercise
Beqembi	lecanemab-irmb	06-Jan-23	Yes	New active substance	To treat Alzheimer's disease
Briumvi	ublituximab-xiyy	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	Enacapavir	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
Brazati	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
Mezlidhia	Blutasidenib	01-Dec-22	No		To treat adults with relapsed or refractory acute myeloid leukemia with a susceptible isocitrate dehydrogenase-1 (IDH1) mutation
Elield	Elplizumab-mzvv	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
Bytgobi	futibatinib	30-Sep-22	No		To treat intrahepatic cholangiocarcinoma harboring fibroblast growth factor receptor 2 (FGFR2) gene fusions or other
Relyvrio	sodium phenylbutyrate/taurursodine	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
Bamzys	mavacamten	28-Apr-22	Yes	Submitted January 2022	To treat certain classes of obstructive hypertrophic cardiomyopathy
Wivjoa	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 relatlimab-rmbw	18-Mar-22	Yes	New active substance	To treat unresectable or metastatic melanoma
Platmy	ganaxolone	18-Mar-22	No		To treat seizures in cyclin-dependent kinase-like 5 deficiency disorder
Wonjo	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	Etumilimab-jome	04-Feb-22	No		To decrease the need for red blood cell transfusion due to hemolysis in cold agglutinin disease
Wabysmo	faricimab-svoa	28-Jan-22	Yes	NOC May 2022	To treat neovascular (wet) aged-related macular degeneration and diabetic macular edema
Kimtrak	tebentafusp-tebn	25-Jan-22	Yes	Issued NOC June 2022	To treat unresectable or metastatic uveal melanoma
Cibingo	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
Quviviq	daridorexant	07-Jan-22	Yes	Submitted October 2021	To treat insomnia
Adbry	Etalokinumab-ldrm	27-Dec-21	Yes	Issued NOC in October 2021	To treat moderate-to-severe atopic dermatitis
Eqvio	inclisiran	22-Dec-21	Yes	NOC July 2021	hypercholesterolemia or clinical atherosclerotic cardiovascular disease as an
Vyvgart	efgartigimod alfa-fcab	17-Dec-21	Yes	New active substance	To treat generalized myasthenia gravis
Tezspire	tezepelumab-ekko	17-Dec-21	Yes	Submitted October 2021	To treat severe asthma as an add-on maintenance therapy
Livtency	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
Voxzogo	vosoritide	19-Nov-21	No		To improve growth in children five years of age and older with achondroplasia and open epiphyses
Besremi	ropeginterferon alfa-2b-njft	12-Nov-21	No		To treat polycythemia vera, a blood disease that causes the overproduction of red blood cells
Scemblix	asciminib	29-Oct-21	Yes	Submitted August 2021	To treat Philadelphia chromosome-positive chronic myeloid leukemia with disease that meets certain criteria
Tavneos	avacopan	07-Oct	Yes	Submitted May 2021	To treat severe active anti-neutrophil cytoplasmic autoantibody-associated vasculitis (granulomatosis with polyangiitis)
Evmarli	maralixibat	29-Sep-21	Yes	Priority Review Jan. 2023	To treat cholestatic pruritus associated with Alagille syndrome
Qulipta	atogepant	28-Sep-21	Yes	Submitted July 2021	To prevent episodic migraines
Wvdak	Etotumab vedotin-tftv	20-Sep-21	No		To treat recurrent or metastatic cervical cancer with disease progression on or after chemotherapy
Exkivity	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
Skytrofa	Enapegsomatropin-tcgd	25-Aug-21	No		To treat short stature due to inadequate secretion of endogenous growth hormone
Korsuva	difelikefalin	23-Aug-21	Yes	Submitted October 2021	To treat moderate-to-severe pruritus associated with chronic kidney disease in certain populations
Wefireg	belzutifan	13-Aug-21	Yes	Submitted September 2021	To treat von Hippel-Lindau disease under certain conditions
Nexvazyme	avalglucosidase alfa-ngpt	06-Aug-21	Yes	Submitted November 2020	To treat late-onset Pompe disease
Waphelo	anifrolumab-fnia	30-Jul-21	Yes	Submitted December 2020	To treat moderate-to-severe systemic lupus erythematosus along with standard therapy
Bylvay	odevixibat	20-Jul-21	Yes	Being reviewed under the Priority Review Policy	To treat pruritus
Wezurock	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
fexinidazole	fexinidazole	16-Jul-21	No		To treat human African trypanosomiasis caused by the parasite Trypanosoma brucei gambiense
Werendia	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

					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
Bylaze	asparaginase erwinia chrysanthemi (recombinant)-rywn	30-Jun-21	Yes	Submitted September 2021.	
Aduhelm	aducanumab-awwa	07-Jun-21	Yes	Submitted June 2021	To treat Alzheimer's disease
Brexafemme	ibrexafungerp	01-Jun-21	No		To treat vulvovaginal candidiasis
Lybalvi	olanzapine and samidorphan	28-May-21	No		To treat schizophrenia and certain aspects of bipolar I disorder
Bruseltiq	infigratinib	28-May-21	Yes	Being reviewed under the Notice of Compliance with Conditions Guidance	To treat cholangiocarcinoma whose disease meets certain criteria
Lumakras	sotorasib	28-May-21	Yes	Being reviewed under the Notice of Compliance with Conditions Guidance	To treat types of non-small cell lung cancer
Rybrevant	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
Bynonta	encastuximab tesirine-lpyl	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
Relbree	viloxazine	02-Apr-21	No		For the treatment of attention deficit hyperactivity disorder
Regalogue	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
Ebtivda	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	Yes	Issued NOC in Sept. 2023	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
Epmetko	tepotinib	03-Feb-21	Yes	Submitted December 2020	To treat non-small cell lung cancer
Bupkynis	voclosporin	22-Jan-21	No		To treat lupus nephritis
Babenuva	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-ggpk	25-Nov-20	No		To treat high-risk refractory or relapsed neuroblastoma
Encivree	betmelanotide	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
Bxlumo	lumasiran	23-Nov-20	Yes	Submitted August 2021	To treat hyperoxaluria type 1
Zokinvy	lonafarnib	20-Nov-20	No		To treat rare conditions related to premature aging
Wekury	remdesivir	22-Oct-20	Yes	NOC July 2020	FDA: To treat COVID-19 Antivirals for systemic use HC:
Imazeb	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
Blenrep	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
Bojolti	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	inebilizumabcdon	11-Jun-20	No		To treat neuromyelitis optica spectrum disorder
Artesunate	artesianate	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	selpercatinib	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-jimr	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)
Ubrelyv	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-efv	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	brolucizumab-dblb	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
TOTAL:				102 HC/197 FDA	

x

<https://www.canada.ca/en/health-canada/services/drug-health-product-review-approval/submissions-under-review.html>

Note: Due to the pandemic, there were some delays in FDA Plant Inspections and product approvals.

See: <https://www.bloomberg.com/news/articles/2021-05-05/pandemic-delayed-some-fda-plant-inspections-product->