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Strategic Policy Branch
Health Canada
Brooke Claxton Building
70 Colombine Driveway
Ottawa, ON K1A 0K9**Consultation on National Strategy for High-Cost Drugs for Rare Diseases**

Novartis is pleased to provide our input into this important consultation on developing a rare disease strategy in Canada. We welcome this initiative to consult a broad range of stakeholders from patients to manufacturers. This is an important step forward to develop an optimal approach to provide timely access to rare disease medicines to Canadian patients. We trust our comments are helpful and we encourage you to contact us should you have any additional questions with our submission.

Overview

Novartis AG is a leading international healthcare company focused on providing solutions to address the evolving needs of patients in societies. We are reimagining medicine and bringing forward transformational therapies as well as utilizing digital technologies to improve the health of societies around the world.

In Canada, we currently employ over 1600 associates located from coast to coast, in our two divisions: Innovative Medicines (“Novartis Pharmaceuticals”) and our cost saving generic medicines division (“Sandoz”). We are at the forefront of bringing new innovations as well as cost saving medicines including biosimilars to Canadians. We are leading the way in advancing new technologies and breakthrough treatments and are the first manufacturer to bring to market both cell and gene therapies in Canada.

In addition to bringing transformational medicines to Canadians, Novartis is committed to supporting the society in which we operate. In addition to our research and development including the clinical trial research we conduct in every region throughout Canada, we are also investing in new digital platforms to bring health care solutions forward to Canadians and around the world. On October 21st 2020, we virtually opened our Novartis BIOME site in Montreal. The Novartis BIOME is the first health tech platform of its kind in Canada, and will enable the brightest minds in healthcare, data science, technology and artificial intelligence to converge. Further, we are focused on our role in addressing the global climate challenge and we have set ambitious targets to be carbon neutral in our own operations by 2025 and across our value chain by 2030. We know industry leadership is required to reduce pollution in Canada and around the globe and this is a priority for Novartis.

Novartis works with the patient community in Canada and around the world on reimagining medicine to improve and extend people's lives. We are committed to partnering with patient organizations and healthcare systems as detailed in our Commitment to Patients and Caregivers¹. We believe that incorporating the patient perspective in our decision-making throughout the product lifecycle can help us to develop better medicines for the benefit of patients and caregivers.

Novartis Leadership in Cell and Gene Therapies

Novartis is a leader in developing highly specialized and targeted treatments for society. Within our broad portfolio we help provide support to a number of rare diseases. Notably, cell and gene therapies are an exciting new generation of medicines with the potential to replace chronic therapies with one-time treatments that can bring significant benefits to specific and targeted groups of patients. We have first-hand experience in addressing the challenges involved in introducing these transformational treatments to health care systems that are not yet prepared to readily adopt such technologies.

In 2019, we launched the first CAR-T therapy (Kymriah®) in Canada, a new approach to treating cancer. CAR-T therapy is the embodiment of personalized medicine, whereby the treatment is manufactured for each patient using the patient's own T-cells, a type of white blood cell. The patient's T-cells are removed and then modified to create an individual one-time treatment for each patient.

This past year, during the COVID-19 pandemic, we received regulatory approval from Health Canada for the first-ever gene replacement therapy (Luxturna®) for the treatment of vision loss due to inherited retinal disease. More recently we received approval for the first gene replacement therapy for the treatment of spinal muscular atrophy (Zolgensma®). These therapies replace the damaged or mutated gene with a new functioning copy and can be life changing for patients and caregivers.

Provincial health care systems that have already begun to adopt these new technologies recognize the value they bring to their citizens and to the system as a whole. Novartis has worked collaboratively with all levels of government to ensure Cell and Gene Therapies are made available to Canadians in a timely manner. Despite the lack of formal pathways in place for these transformational treatments, there has been a willingness by all parties to find timely solutions to ensure Canadians can access them.

As the Government of Canada moves towards a more formalized approach to supporting access to rare disease treatments across the country, timeliness and a sense of urgency will be key. We must continue to actively work together to ensure Canadian patients do not have to suffer or wait for access, while at the same time enabling the provinces to have the autonomy, flexibility and funding to deliver this specialized care.

Given our unique experience as the first manufacturer to launch Cell and Gene Therapies in Canada, and our overall commitment to delivering innovative treatments for rare diseases, we welcome this consultation and the opportunity to share our insights and learnings for consideration.

We have outlined six **Key Principles** that encapsulate our vision for a new rare disease framework for Canada and are bolstered by five concrete recommendations for bringing it to life. It is our hope

¹ <https://www.novartis.com/our-focus/patients-caregivers/novartis-commitment-patients-and-caregivers>

that with these principles and recommendations, we can contribute to an overarching goal of improving patient outcomes.

Key Principles

- 1. Predictable funding for the provinces and territories.** Given the responsibility of the provinces and territories in the delivery of health care in Canada, they should be able to count on increased and sustainable financial support from the federal government via health transfers to provide timely access to rare disease treatments on a per capita basis.
- 2. Respect and support health care delivery in all jurisdictions.** Respecting the principles of the Canada Health Act, support must be provided to provinces and territories in their role to deliver health care in a way that allows for regionalization and flexibility in the delivery of rare disease programs based on regional needs.
- 3. Efficient and agile process.** By definition, future treatment options for rare diseases will take different forms that may not suit the current processes in place. It is important that the drug approval and reimbursement processes for rare diseases can be both agile and fast to offer relevant options to patients in a timely manner.
- 4. Equity.** In the interests of fairness, it is imperative that the process that will be put in place for rare diseases does not differentiate in terms of the type of coverage for rare oncology drugs and the type of coverage for rare non-oncological diseases. In addition, patients regardless of provincial jurisdiction, need to have access to comparable treatment options.
- 5. Value.** Recognition of both the therapeutic and economic value of rare disease therapies is critical. The societal impact must be considered to fully integrate the reality of the health and economic burden that those living with and affected by rare diseases have to face. This requires more inclusive methods to take into account several dimensions.
- 6. Responsibility.** Governments, drug manufacturers and various stakeholders are collectively responsible for ensuring the success of a drug access program for rare diseases that considers the invaluable insights of patients and caregivers. Authorities must ensure that the process is fair, swift and efficient. Evaluation processes should engage appropriate expertise, use methodologies tailored to rare disease populations and novel therapies and commit the right level of resources to respond quickly to requests for both the therapeutic and the economic evaluation of drugs for rare diseases. Manufacturers have a responsibility with regard to the adequate and optimal use of drugs, to respect any new processes developed and to inform as much as possible in advance of the products in development that may be submitted for approval in Canada.

Recommendations – to ensure All Canadians have access to New Technologies and Treatments for Rare Disease

1. Strengthened Screening Programs and Patient Registries

The Government of Canada, through the development of a National Rare Disease Strategy should support and promote advanced screening and diagnostic programs, as well as the

dissemination of knowledge among physicians, patients and caregivers to support and improve the identification and treatment of rare diseases. National standards for newborn screening panels, improved access to genetic testing and counselling services, and consideration for cutting-edge diagnostics like whole genome sequencing would enhance and improve overall patient outcomes. The establishment and maintenance of national registries to support the ongoing management of rare diseases across the country would encourage greater interprovincial collaboration and knowledge sharing including among healthcare providers, who have a critical role to play in supporting patients and are therefore critical to the success of any rare disease program.

2. Greater alignment between Regulatory, HTA and Payors (PCPA)

We applaud and encourage the continuation of aligned reviews between Health Canada and HTAs that was introduced under R2D2 with the goal of reducing the time between Health Canada's market approval and HTA funding recommendations. Novartis participated in early pilots of this approach, and now authorizes the exchange of information between Health Canada and HTAs for all of our submissions. While these efforts have demonstrated a reduction in the time to HTA recommendations, continued efforts to reduce time to final reimbursement are needed. Provisional negotiations with payers should take place in parallel to reviews by Health Canada and HTAs to truly expedite access for patients. For rare disease medications, where time to access is even more paramount we believe this would help put the patient more at the center of these review processes, and expedite decision making through greater collaboration.

3. Agile Regulatory Framework

Health Canada has demonstrated immense agility in its management of the review of vaccines and treatments for COVID-19. Some of the measures introduced through these interim orders should also be considered in the review of submissions for treatments of rare diseases, specifically: The use of more agile administrative and application requirements for authorizing new drugs, such as rolling reviews. In addition, Health Canada should have the ability to leverage approvals from trusted foreign regulators in authorizing new drugs. Such measures would encourage manufacturers to bring treatments for rare diseases to Canada, as well as expedite the availability of treatments for these conditions. Health Canada's experience with joint regulatory reviews (e.g. Project ORBIS) is a great initiative that supports timely access and efficiencies and we support continuation of these endeavours.

4. Value-Based Approach

We believe all stakeholders need to work together to ensure new innovations are accessible and affordable to health care systems and suggest that a value-based procurement approach is one that addresses these needs. Clinical value, patient value and health care system value together with societal value should be part of the decision making process for all therapies, but particularly for rare disease treatments. Given our experience in launching first in class therapies for rare diseases, and the expertise that the provinces have utilized to access new treatments, we believe that as more advancements in health care technologies are made a value based procurement model needs to become the gold standard approach.

We encourage provincial and territorial jurisdictions as well as HTA authorities to utilize a value-based approach for the reimbursement of all new pharmaceuticals entering the market. In addition, we believe any new process needs to be flexible and be adaptable to new

innovations that enter the market. A more nimble approach by all authorities, will encourage earlier launches of break through treatments in Canada.

5. Respect Autonomy of Provinces and Territories to Encourage Innovation

Any new pharmaceutical program for Canadians, including a rare disease strategy, needs to respect the autonomy of the provinces and territories as the deliverer of health care and must ensure that the new program remains flexible to their program needs. The management of rare diseases is intrinsically linked to the organization of care and the medical practices that take place in the provinces. This is why the provinces must be in charge of the acceptance of drugs used for rare diseases in their respective health systems.

Additional funding for rare disease programs should be made available by the federal government through the Canada Health Transfer and provided to the provinces to enhance and strengthen their abilities in providing access to care for patients with rare diseases. Additional funding should ensure provinces and territories deliver more timely health care to patients with the goal of access to new treatments at the same time as regulatory approval.

The Future

Bio-science and technology are fostering the development of new breakthrough medical treatments at an accelerated pace. In addition, with the advancement in data technologies the future of health care for societies in addressing unmet medical needs has never been greater. These advancements are helping patients in some of the most challenging areas including sickle cell disease, cystic fibrosis, spinal muscular atrophy and retinal diseases. Further, the COVID-19 pandemic and the rapid development of vaccines and other treatments has demonstrated what is possible when human resilience and collaborative science rise to the occasion.

Novartis is committed to deliver on our purpose to reimagine medicine by developing transformative new treatments and finding innovative solutions to the world's most pressing health care challenges. Recently, we announced collaborations with other manufacturers to utilize our capabilities and manufacturing sites to support the rapid production of COVID-19 vaccines to support the global demand for these treatments. We are proud to step up and work collaboratively in addressing this global pandemic and work with other partners and we are open to future partnerships with all stakeholders to accelerate access to innovations for patients in need.

We encourage the Government of Canada through this consultative process to embrace innovation and find ways to support the provinces in making room for new technologies in a timely manner to improve the lives of patients and caregivers touched by rare disease and all Canadians.

Sincerely yours,

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