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SUBMISSION

Mitsubishi Tanabe Pharma Canada Inc. (MTP-CA)

National Strategy for High-Cost Drugs for Rare Diseases Consultation

INTRODUCTION

Mitsubishi Tanabe Pharma Canada Inc. (MTP-CA) is the Canadian affiliate of Mitsubishi Tanabe Pharma Corporation (MTPC), a Japan-based multinational with a history stretching back over 300 years. Over our history, the company has discovered several distinctive treatments — considered first-in-class at launch — for serious diseases including diabetes (DM) and multiple sclerosis (MS). While these successes represent a strong part of our identity, we continue to build on that legacy by seeking new solutions to the pressing needs of patients. Our mission is "creating hope for all those facing illness." Our vision is to provide optimal therapy to each individual in need.

MTP-CA was established in 2018 with the goal of providing therapies for some of the most difficult-to-treat diseases, including amyotrophic lateral sclerosis (ALS), a progressive and incurable neurodegenerative disease. Our first product, RADICAVA® (edaravone) for the treatment of amyotrophic lateral sclerosis (ALS) was approved by Health Canada in October 2018. Based on prevalence, ALS falls within the commonly accepted definition of rare disease in those jurisdictions where such designation is available.

Since 2018, MTP-CA has made substantial investments in the Canadian healthcare system, including supporting treatment for over 200 Canadian ALS patients through our best-in-class patient support program (PSP) and authorized by Health Canada's Special Access Program (SAP). Our PSP also provides turnkey support to the Canadian ALS communities throughout the patient journey and addresses an important gap within existing healthcare systems, including supporting treatment infusion. The existence of this important PSP was a critical treatment support for Canadian ALS patients, given the considerable gap between Health Canada approval in October 2018 and eventual reimbursement across public and private plans in Canada.

Our experience in bringing RADICAVA® to Canada has shaped our perspective on the gaps in Canada's current approach to ensuring access to necessary treatments for rare disease patients.

CONSULTATION FEEDBACK

MTP-CA is an active member of both BIOTECanada and RAREi (Canadian Forum for Rare Disease Innovators), and fully endorses their submissions to this consultation. In particular, we would emphasize the following key aspects to Health Canada as you consider future policy approaches in this important, but too often neglected, area of our healthcare system.

1. A holistic policy approach that recognizes uncertainty.

The consultation document prepared by Health Canada raises a number of key issues facing rare disease patients and a system attempting to balance access to therapy with sustainable budget considerations. As Health Canada has rightly identified, Canada is falling short of international best practices in terms of attracting a higher share of globally available therapies and providing timely, equitable reimbursement coverage for Canadian rare disease patients.

The consultation document places a disproportionate and narrow emphasis on the cost of rare disease therapies without sufficient attention to disease severity and the value of innovative therapies against any available standard of care. Many rare diseases have few if any available treatment

options, and as such place a substantial burden on patients, families, caregivers, and the broader healthcare system. Questions of cost of therapy must be placed in their proper context.

Further, it is broadly recognized that Health Technology Assessment (HTA) review methodology is not well suited for assessing rare disease therapies. More appropriately designed evaluation methods to rare disease drugs should be considered to enhance HTA review processes such as emphasize less on pharmacoeconomic analyses and more on patient outcomes and clinical value in clinical conditions for which there are currently no or very limited treatment options. Focus should be on budget impact including predictability versus cost effectiveness.

In our view, it is important to acknowledge the inherent uncertainty in the larger rare disease space as a function of smaller patient populations and limited disease understanding. Within Canada as a whole, let alone individual Provinces and Territories, medical knowledge and experience may be concentrated in a small number of specialists and centres. Access to screening and testing is uneven and inconsistent on a national basis. This translates to challenges in monitoring and establishing patient diagnosis for many rare diseases, with variability in time-to-diagnosis and overall patient experience.

Once diagnosed, many patients experience uncertainty with respect to access to treatment, including the availability and criteria for individual therapies but also critical related issues such as access to health professionals, infusion services, and other important elements to their care.

A much more integrated approach to rare disease policy is required to coordinate these various elements which make up the rare disease patient experience in Canada. Limited or incomplete reforms to aspects of the process are unlikely to be sufficient to improving Canada's overall management of rare diseases in a sustainable manner.

2. A more flexible, specific and value-based management pathway.

A number of the consultation questions begin to address potential elements of an effective Canadian strategy for rare disease. The current Canadian medicines review and reimbursement system is complicated and includes multiple stages, agencies, processes, and timelines. Without introducing further complication, a rare-specific approach can be set forth building on the foundation of the existing system and supported by targeted policy reforms. There are two particular areas for priority focus:

- **Improving Evidence Generation**. Evidence limitations within many rare diseases are unavoidable, but key steps are available to policymakers to help address this challenge. Canada's overall infrastructure for disease registries and data management is not yet at the leading global standard. With coordinated investment and reforms, various national and Provincial/Territorial resources and stakeholders can be combined to improve efficiencies and support better evidence generation and analysis. Further, Canada can take additional steps to improve its level of integration with global registries and initiatives. This is a critical consideration given the global nature of medical science and the highly limited patient populations for many rare diseases.
- **Enabling Value-Based Agreements**. In cases where a therapy presents greater levels of uncertainty for all stakeholders, including reimbursement authorities, it can be challenging to find a mutually acceptable pathway to ensure timely patient access to treatment. The current focus of the Canadian process tends towards emphasis on evaluation and negotiation at the point of market entry, which can translate into delays and unpredictability for all parties. Globally, there has been significant progress in implementing more dynamic, value-based agreements. These can incorporate the expectation of ongoing evidence generation with reassessments at set intervals.

Reimbursement terms and conditions, including price, can be linked to clinical outcomes in the real world over time. Health Canada could play a leadership role in providing the resources and tools to enable greater use of these agreements. Canada has been relatively slow to embrace these approaches, and there is an immediate opportunity to pursue in the context of rare diseases, which offer a much more manageable scope.

We appreciate Health Canada's consideration of this submission and those of our trade associations. MTP-CA remains committed to building a better healthcare system and to supporting the patient communities we serve. Canada remains behind other jurisdictions in not having a clearly defined and tailored rare disease policy. There is greater urgency to address this challenge, given the number of treatments still not available in Canada and the exciting potential for future treatment options emerging from medical research.

MTP-CA would welcome any future opportunity to contribute to the formation of improved public policies in support of Canadian rare disease patients – please do not hesitate to reach out to me directly in this regard.

Sincerely,

Andy Zylak
Country Manager
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