A Wave of Innovative Rare Disease Drugs is Coming – Is Canada's Healthcare System Ready?

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Takeda partnered with IQVIA to develop a report evaluating Canada's Health System Readiness for the DRDs in the pipeline

Rare Diseases in Canada

- Rare diseases impact **1 in 12 Canadians** and are often **life threatening**
- Novel drugs for rare diseases (DRDs) are crucial for addressing unmet needs, with over **1,100 DRDs** in development and nearly **200 expected to launch in Canada** within the next **10 years**
- Canada's healthcare system must be prepared to effectively support this increase in DRDs **to facilitate timely access to patients**

Report's Objective

- To assess the DRDs expected to launch in Canada within the next decade and discuss the policy considerations for the Canadian healthcare system.

To identify areas of need, DRDs obtained from IQVIA™ Pipeline Intelligence database were segmented using various criteria

Identification of DRD Selection

- IQVIA™ Pipeline Intelligence database was used to identify DRDs in the drug development pipeline.

- DRDs from Pipeline Intelligence were identified based on orphan drug status, or those that had an indication for a rare disease.

Data Segmentation Methodology

- Results were analyzed using the following stratifications:
  - Phase of drug development
  - Therapeutic area
  - Route of administration
  - Therapy type
  - Rare versus ultra-rare disease status
  - Pediatric indications
  - Requirement for genetic testing
  - First-in-class treatments

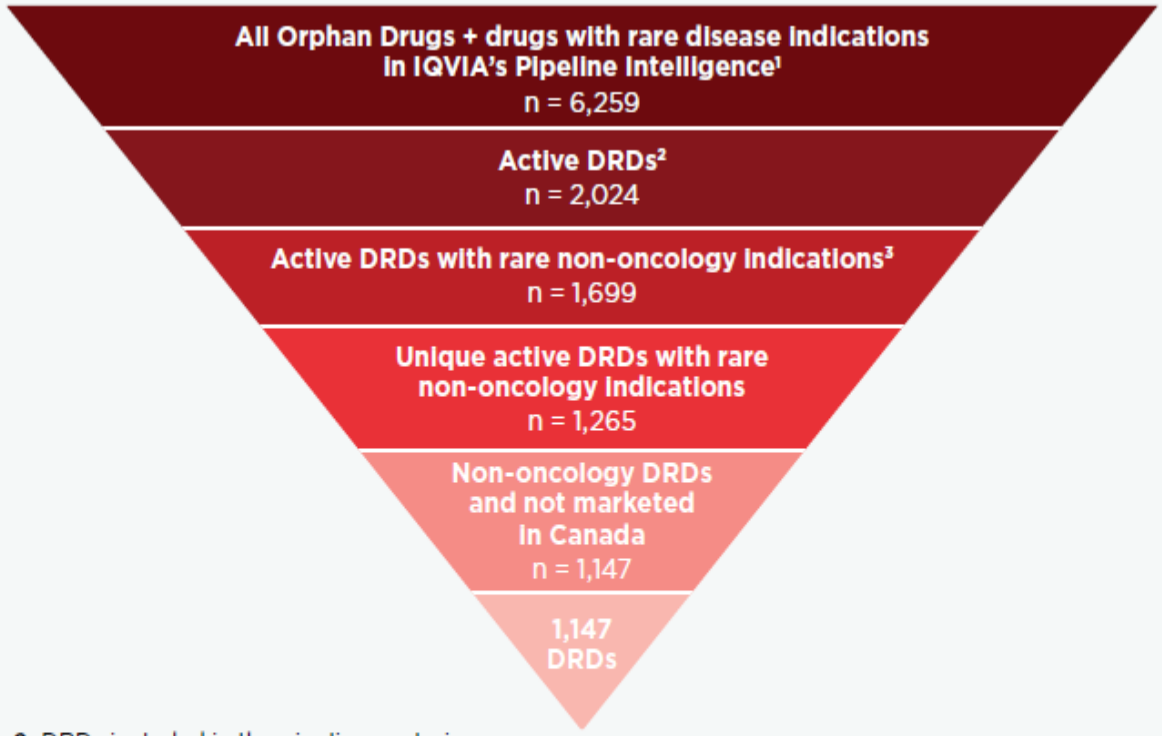


Figure 2: DRDs included in the pipeline analysis

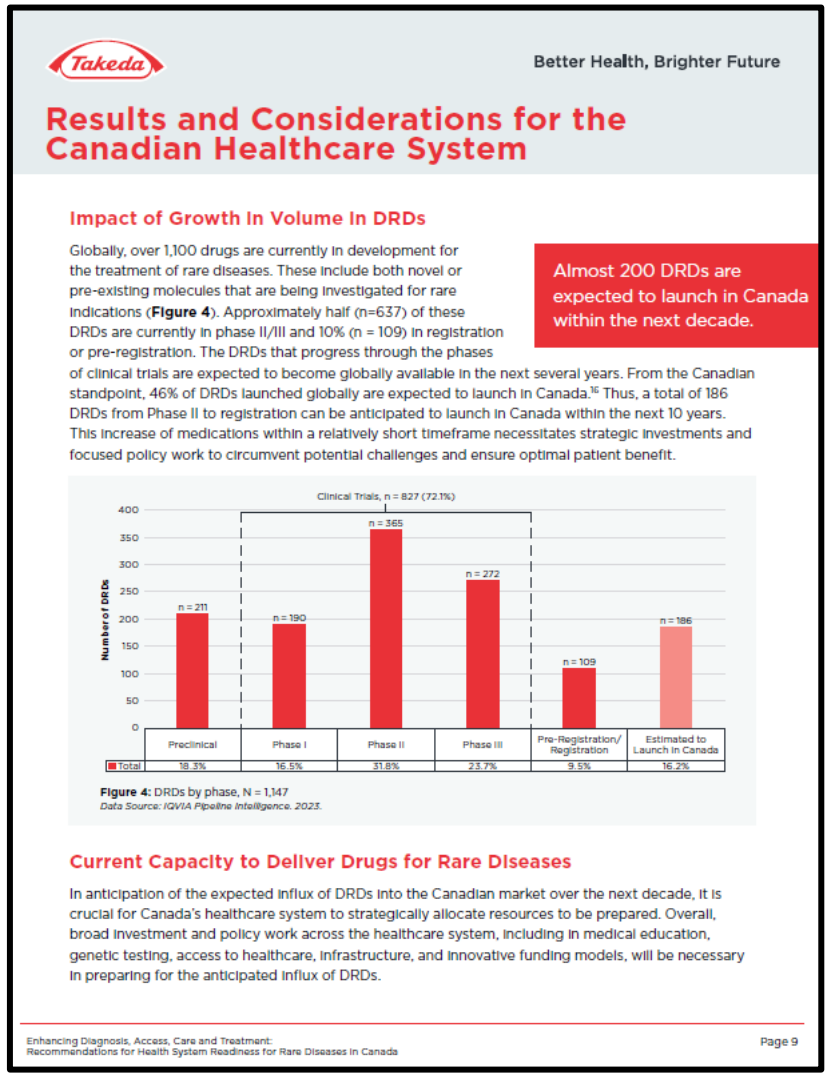
1. Includes drugs being developed for rare diseases and/or have been granted orphan drug status.
2. Active status is applied to all drugs in Discovery phase to Registered where significant research and development activity has been reported within the last three years; Marketed drugs where the drug is still in a pre-marketed development somewhere in the world or for additional indications or the drug has been launched worldwide within the last three years.
3. Drugs with only oncology indications or drugs with oncology and non-oncology indications, but the non-oncology indication is not considered rare.

Data Source: IQVIA Pipeline Intelligence. 2023.

There are over 1,100 DRDs in the pipeline, with many requiring specialized medical education, storage requirements, and diagnostics

Key Findings

- There are **over 1,100** DRDs currently in the pipeline.
- Roughly **39% of all DRDs are first-in-class.**
- The DRDs are distributed across a diverse set of therapeutic areas, with a substantial proportion targeting conditions related to the **nervous system, immune system, and alimentary tract/metabolism.**
- Approximately **25% of DRDs** have indications for genetic conditions.
- Over **50% of DRDs** can be used to treat **pediatric** conditions.
- Over **25% of DRDs** in the pipeline treat an **ultra-rare** disease.
- Approximately **33% of DRDs** are administered via intravenous **infusion** or **specialized injection**
- Within the pipeline, **51% of DRDs are biologics, blood products, and next-generation biotherapeutic**s, which include gene, cell, nucleotide-based, and RNA-based therapies



Takeda — Better Health, Brighter Future

Results and Considerations for the Canadian Healthcare System

Impact of Growth in Volume in DRDs

Globally, over 1,100 drugs are currently in development for the treatment of rare diseases. These include both novel or pre-existing molecules that are being investigated for rare indications (**Figure 4**). Approximately half (n=637) of these DRDs are currently in phase II/III and 10% (n = 109) in registration or pre-registration. The DRDs that progress through the phases of clinical trials are expected to become globally available in the next several years. From the Canadian standpoint, 46% of DRDs launched globally are expected to launch in Canada.[16] Thus, a total of 186 DRDs from Phase II to registration can be anticipated to launch in Canada within the next 10 years. This increase of medications within a relatively short timeframe necessitates strategic investments and focused policy work to circumvent potential challenges and ensure optimal patient benefit.

Almost 200 DRDs are expected to launch in Canada within the next decade.

Figure 4: DRDs by phase, N = 1,147
Data Source: IQVIA Pipeline Intelligence. 2023.

Current Capacity to Deliver Drugs for Rare Diseases

In anticipation of the expected influx of DRDs into the Canadian market over the next decade, it is crucial for Canada's healthcare system to strategically allocate resources to be prepared. Overall, broad investment and policy work across the healthcare system, including in medical education, genetic testing, access to healthcare, infrastructure, and innovative funding models, will be necessary in preparing for the anticipated influx of DRDs.

Enhancing Diagnosis, Access, Care and Treatment: Recommendations for Health System Readiness for Rare Diseases in Canada — Page 9

DRDs – Drugs for Rare Diseases
Findings are based on the number of DRDs in Phase III, registration, or pre-registration (n = 381)

Six key recommendations were made in Takeda's Health System Readiness Report

Recommendations for Health System Readiness of DRDs

The Need for Medical Education

While education is essential for all rare diseases, particular attention should be given to first-in-class DRDs, which offer novel treatment opportunities. Education for physicians and other healthcare providers will be key to ensure these treatments reach patients.

The Role of Genetic Testing

Focus on an equitable pan-Canadian approach to investment in, and implementation of, genetic testing (including newborn screening and testing during childhood and beyond) across Canada.

Delayed Diagnosis and Access Challenges

Investment and policy development that reduces barriers for those living in rural or underpopulated regions is imperative to facilitate healthcare access for all Canadians affected by rare diseases.

Address Infrastructure Challenges

As Canada prepares for an influx of DRDs, the demand for cold chain storage and transport may rise. This critical infrastructure requires investment, policy work and the development of protocols to prevent medication waste.

Adopt Innovative Funding Models and Increase Collaboration

Collaboration will be key to ensuring access to treatments for patients with rare diseases. Innovative funding models, including amortization over time, OBAs and modifying HTA requirements, will be important in ensuring patients can access treatment.

Patient Engagement and Partnership

Patient participation is crucial to ensuring that appropriate care reaches patients. Collaborating with patient groups throughout the healthcare planning process is vital to meeting the needs of patients with rare diseases.

Discussion & Questions