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An Orphan Drug Framework for Canadians

Presented to:

Aplastic Anemia and Myelodysplasia
Association of Canada

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Canada

Introduction – Orphan Drug Framework

- Health Canada is developing a federal regulatory framework for drugs for rare diseases (orphan drugs).
- This framework is intended to address the **unique challenges** of studying small patient populations.



Why move on orphan drugs now? (1)

Current Priorities: Guided by Canadian government initiatives to gain efficiency and sustainability [Regulatory Cooperation Council (RCC) and Red Tape Reduction Commission (RTRC)].

Current Regulations: Inflexible, out-dated and lacking appropriate tools.

Focus on Rare Disease Patients: Need to improve access to orphan drugs without compromising patient safety.



Why move on orphan drugs now? (2)

Drug Development: Undergoing radical changes and has shifted away from blockbuster to targeted therapies due to:

- Rapid advances in science
- Better understanding of disease
- Plummeting costs of genetic testing



What do we mean by “Framework”?

The proposed Orphan Drug Framework will encompass:

Regulatory Amendments: To the *Food and Drug Regulations* in the form of a new division specific to orphan drugs.

Supporting Guidance: To facilitate information flow to and from Health Canada.

Supporting Operational Structure and Processes: To gain the greatest efficiencies and enable a high degree of collaboration and information sharing.



Orphan Drug Framework - Key New Features(1)

Life-cycle Approach: To take into account a wide body of evidence before and after a drug is marketed.

Orphan Drug Designation: Criteria and processes that are aligned with those of the US and EU so collaboration is possible.

Regulatory Advice: Formal advice by Health Canada or in conjunction with international regulators.

Expert Advice and Patient Representation: To support the best possible decision making.



Orphan Drug Framework -Key New Features(2)

Market Authorization: “Licence” to sell which could be amended, reassessed, suspended or cancelled.

Post-Market Authorization Plan (pMAP): To support ongoing assessment and management of benefits, harms and uncertainties associated with the drug.

Ability to Obtain Information: Flexible processes to adjust to different situations in support of reassessments.

Transparency: Information sharing throughout the lifecycle of the drug.



Orphan Drug Framework - Life-cycle Approach

“What is known about the **benefits, harms and uncertainties** associated with a drug changes over time”

Current:

Limited point-in-time oversight based on applications for clinical trials and marketing authorizations.

Future:

Expanded and continuous beginning at early development stage with medical plausibility and encompassing a greater ability to define post-approval information gathering.



Orphan Drug Framework - Designation

Criteria: Applicants would have to demonstrate: prevalence in Canada (less than 5 in 10,000), severity of the disease, medical plausibility of the drug and a lack of existing therapy or potential for significant improvement.

Benefits of Designation: Scientific and clinical protocol advice, accelerated review, improved transparency, fee reductions.

First Regulatory Step to Reduce Uncertainty: Designation represents early support by the regulator which may encourage further development of the drug



Orphan Drug Framework – Regulatory Advice

Facilitating conduct of studies and information gathering about the benefits, harms and uncertainties of a drug.

Provided on request: To Orphan Designation holders

In common: Where possible with international regulators.

Including: Opinions of experts and patients when expertise is not available within Health Canada.

Formalized and stable: Unless new information renders previous advice invalid. Ability to amend advice when necessary.



Orphan Drug Framework – Patients and Experts

Facilitating: The use of scientific and medical experts and providing opportunities for the patient perspective.

With over 7000 rare diseases identified, Health Canada recognizes that specialized expertise may be needed to assess certain information in an application.

Patients: Provide valuable insight regarding the severity of a disease, an unmet medical need and can relate experiences with the drug.

International regulators as well as domestic HTAs have been expanding opportunities for patient input.



Orphan Drug Framework – Market Authorization

An approval to sell an orphan drug.

A single Market Authorization (MA): Would be issued and could be amended, reassessed, suspended in whole or in part or cancelled.

Facilitates the regulatory life-cycle management: Of a drug as any changes from the point of initial authorization will be tied back to the MA.

Provides for a “licence” to which terms and conditions could be introduced in contrast to existing Notice of Compliance (NOC) structure.



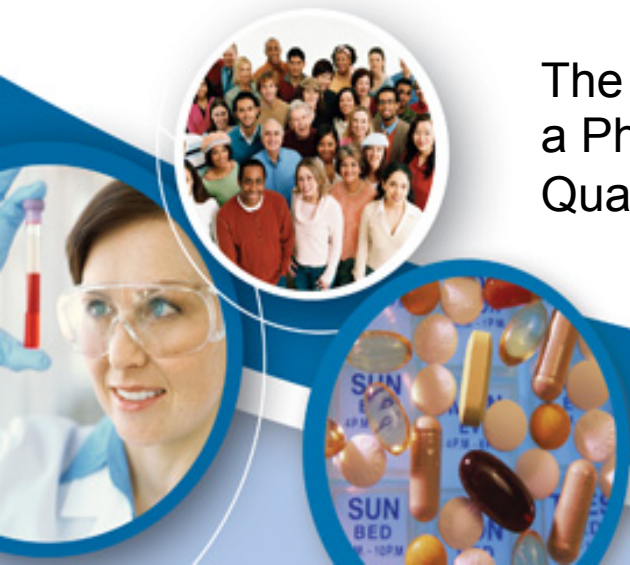
Orphan Drug Framework – pMAP

An “umbrella” **plan** for post-approval management of harms and to reduce uncertainties associated with the drug.

Provided on application: For an MA and tailored to the specific circumstance of the drug.

Adherence: would be a condition of approval.

The plan could include: a Risk Management Plan (RMP), a Pharmacovigilance Plan (PVP), Confirmatory studies, Quality commitments, pre-planned amendments.



Orphan Drug Framework – Ability to Obtain Info

New abilities would be provided to allow Health Canada flexible choices to obtain information about a drug.

Health Canada may receive information regarding the benefits, harms and uncertainties of a marketed drug from a variety of sources including the MA holder.

This new information may trigger a **reassessment** requiring the MA holder to provide information to resolve uncertainties.

The reassessment process could result in no change, an amendment, suspension or cancellation of an MA.



Orphan Drug Framework – Transparency

Expanded transparency at all stages in the life-cycle

Supports regulatory decision-making

Allows involvement by patients, health care professionals, clinicians, HTA and others early stages where appropriate.

Positive or negative decisions and the basis for them would be made public.



Information is the key

“The right information from the right sources at the right time”

This forms the basis of the best regulatory decision making at any point in the life-cycle of a drug.

The new framework will enable the input of information not only from the manufacture of a drug but from experts, patients, health care professionals, international regulatory partners, health technology assessors (HTA) and payers.



Next Steps

- Continue to consult with other decision makers, including Canadians, health care professionals, payers, academia, and industry, as well as international partners.
- Table a new regulatory framework.
- Continue to promote changes in regulatory culture.



Next Steps

Thank You

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