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# Vision for Creating New Therapies for Patients with Rare Diseases:

# View from Industry / GSK

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# Disclosure

Marc Dunoyer is employed by GSK as Global Head of Rare Diseases and Chairman of GSK Japan. He is a member of the Corporate Executive Team. He is a GSK shareholder.

Marc is not a consultant or a paid instructor. He does not receive any grants or research support, nor is he part of the Speaker's Bureau.

# Summary

- Numbers of approvals linked to introduction of orphan drug legislations
  - More recently, no annual increase in number of new drugs for rare diseases coming to market
  - Lack of global recognition (only 6 countries/regions have clear orphan drug policy)
- NEED for novel approaches to regulatory approval to accelerate access

# Changes in Regulatory Approach

- Regulatory Harmonisation
  - Going further …
- Progressive Approval
  - Adopting Innovative Approaches to Drug Development
- Increased Dialogue

# Regulatory Harmonisation

- Harmonised regulatory advice
  - "Joint" or "Tripartite" advice between agencies rather than "Parallel"
- Alignment of detailed requirements necessary
  - e.g. Clinical trial design, endpoints, etc
- "Mutual" Recognition of regulatory review
  - Shared data assessments
- Recognition of orphan drug licences
  - Adopted by countries without an orphan regulatory pathway

# Progressive Approval

- Allows earliest access to new medicines
- In Rare Diseases data generation is a continuum
- Risk-based approach
  - Key role of patient organisations and physicians
- Conditional with further confirmatory development and review
- Positive examples exist

# Increased Dialogue

- Unchartered territory of rare diseases requires more frequent dialogue (formal and informal)
- Between sponsors, regulators and patient organisations
- Generates optimal approaches for clinical development and patient access
- Engagement of political support for regulatory and reimbursement solutions that speed up wider patient access

# Rare Diseases – Opportunities for Acceleration

iscovery | AOP, published online 24 June 2011; doi:10.1038/nrd3493

COMMENT

*Nature Reviews Drug Discovery*

## Accelerating access to treatments for rare diseases

*Marc Dunoyer*

Changes in regulatory policy and legislative incentives to promote the development of drugs for rare diseases — orphan drugs — have led to increases in the number of orphan drug designations, but the rate of such products reaching the market remains frustratingly flat. This article highlights areas in which novel approaches could facilitate regulatory approval and access to treatments for rare diseases.

10 solutions to accelerate access to treatments in Rare Diseases

# Conclusion

- Collaboration in which new regulatory systems must evolve to bring treatments to the market to serve rare disease patients more effectively and more rapidly