NGO COMMITTEE FOR
RARE DISEASES

A Perspective from Industry

Mobilizing All Stakeholders in the Rare Disease Community Worldwide on the Road to 2030

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NGO Committee for Rare Diseases
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A Committee of **CoNGO**

CoNGO
The Conference of NGOs
in Consultative Relationship
with the United Nations

About IFPMA

The International Federation of Pharmaceutical Manufacturers and Associations (IFPMA) represents research-based biopharmaceutical companies and national pharmaceutical industry associations across the world.

KEY AREAS OF WORK

- Advocate policies and practices that encourage discovery of and access to medicines and vaccines
- Facilitate collaboration, dialogue and understanding within the industry and with other key global players in the health community
- Bring all stakeholders together to foster innovation, promote resilient regulatory systems, uphold ethical practices and advocate sustainable health policies to meet global health needs

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The rare disease burden is significant

There are approximately
7,000 rare diseases

Approximately **50%**
affected by rare diseases
are children

40% of rare disease patients are
misdiagnosed more than once

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Specific challenges of rare disease still need to be addressed

* **Little understanding of the diseases** - very little published
* **No precedent** for clinical study design & endpoints
* **Regulatory path is untrodden**
* **Few and hard-to-find patients**, geographically dispersed for study and clinical trial enrollment
* Increased focus on **payers perspective, real world evidence, value demonstration**
* **Intense need for education** of patients, caregivers and physicians
* Extremely high medical need – pressure for **early access**
* **Clinical trial transparency** is important

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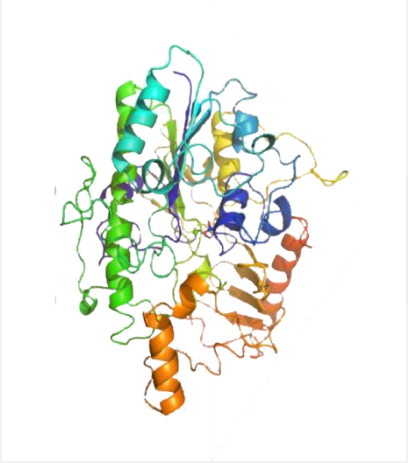
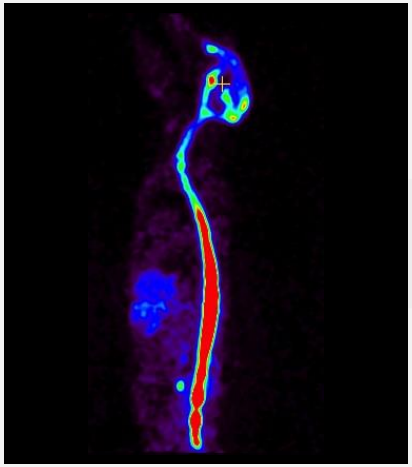
Serial innovation can help… but alone it is not always enough



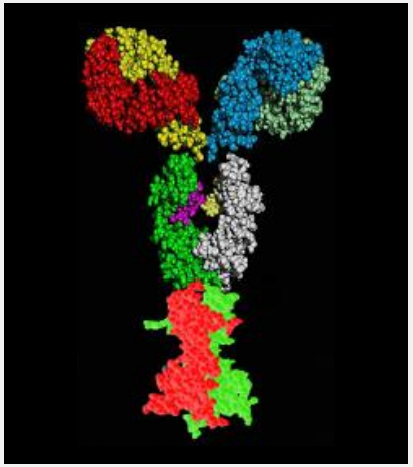
ELAPRASE

- Novel Enzyme Replacement Therapy (ERT)
- IV administration



Hunter IT program*

- Novel CNS delivery device for ERT
- Novel formulation
- Combination product



'Trojan Horse' program*

- Novel delivery approach for ERT
- IV delivery

Coming together to create opportunities for rare disease patients

Scientific innovation

Clinical trial design

Regulatory pathways

Infrastructure & access

Education & awareness

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IFPMA Rare Diseases Working Group

Recognizing the importance of addressing unmet need in rare disease around the world, IFPMA has recently established a Rare Disease Working Group.

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Industry's collective commitment to Rare Diseases

IFPMA aims to support the rare disease community through focused activities in four critical areas.

AWARENESS

Raise awareness about rare diseases and promote as a public health priority

INCENTIVES

Foster discussions on policy incentives and orphan drug regulatory frameworks at local level

PARTNERSHIPS

Build international networks with rare disease stakeholder community

ACCESS

Ensure sustainable patient access to diagnosis, treatment and care

Tangible deliverables for the Rare Diseases community

AWARENESS	Produce and disseminate an IFPMA Educational Brochure to raise awareness of rare diseases among key IFPMA stakeholders at global and country levels
INCENTIVES	Engage actively at local level to enhance rare diseases as a public policy focus and/or promote orphan drug incentive frameworks
ACCESS	Develop IFPMA Policy Principles in Rare Diseases aimed at improving patient access to therapies that treat rare diseases
PARTNERSHIPS	Build awareness amongst key stakeholders (patients, clinicians, policy makers, national industry associations) of the new IFPMA Rare Disease initiative

Thank You