EPTRI

EUROPEAN PAEDIATRIC TRANSLATIONAL RESEARCH INFRASTRUCTURE

WORLD DUCHENNE ORGANIZATION

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Patient centric research in the rare and paediatric diseases

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EPTRI Stakeholders Roundtable 9th July 2020

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Paediatric and Rare Diseases medicines development

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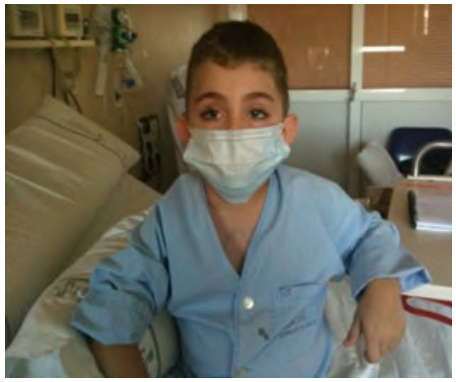
Do we have more research and paediatric products in the pipeline

yes

YES …BUT :



Orphan?
Paediatric Cancers?
Neonates?

Most products remain “adult only”
Regulatory Provisions and Benefits are under-used
R&D Infrastructures and Finance is Fragmented

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Understanding the environment I

Socioeconomic

- **Ageing** of populations leads to high demand for care
- **Challenging economic** environment with direct **negative impact on the budgets**
- The **increasing cost** of the development of an innovative medicine, today estimated at USD 2.6 billion
- The skepticism of the industry and government to **re-investment in basic science**
- **Drop in pharmaceuticals expenditure**

Technical

- **Low RD Productivity**
- **Under usage** of new **IT** technologies, wearable , data platforms etc
- **New challenging** technologies in RD

Understanding the environment II

Political

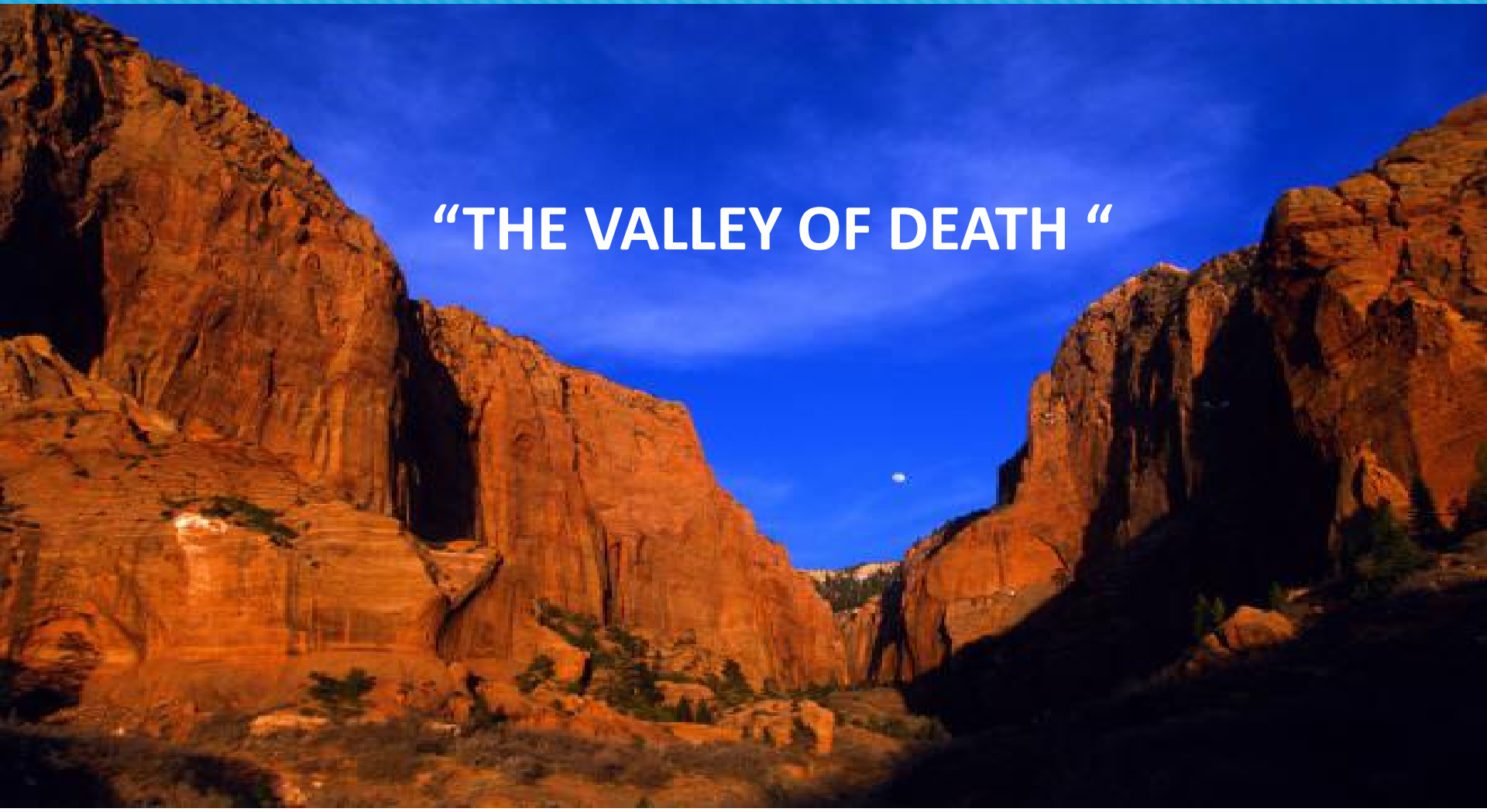
- The organizational **bureaucracy** and **natural resistance** to change
- The limitation of **mandates**

Mental

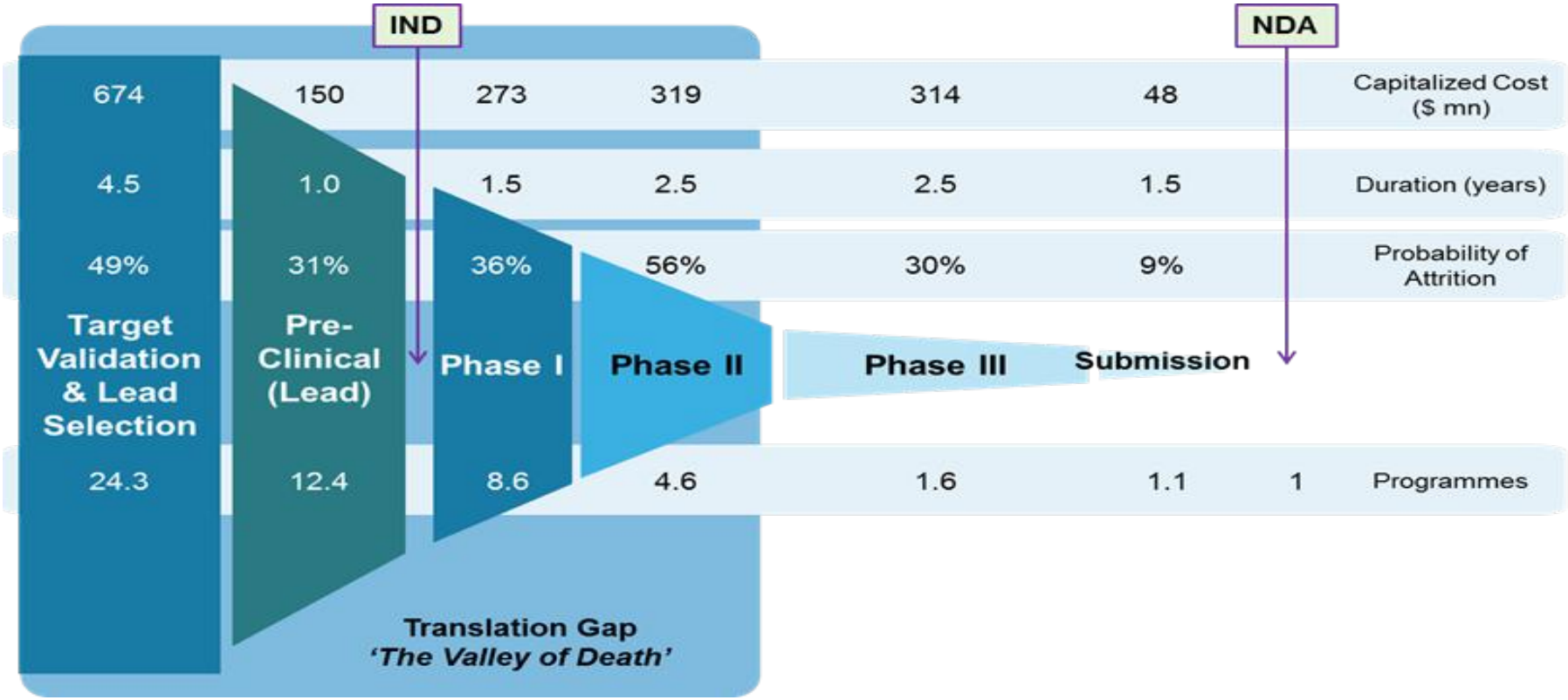
- The **mental barriers** of the healthcare systems themselves
- In **Silo attitude**, unproductive competition mentality, misperceptions and misconceptions.
- **Tunnel vision** in most of the stakeholders

We can not develop medicines the same way we did 50 years a go !

The Results



"THE VALLEY OF DEATH "



Negative productivity growth which **has halved every 9 years since 1950**

Figure 1. Drug development cycle and the 'valley of death'. Schematics of the drug development lifecycle, duration of each one of the stages, capitalized costs and probability of failure. Adapted from {Paul, 2010 #886} and Michael J. Fox Foundation. **Unsustainable in both Worlds**

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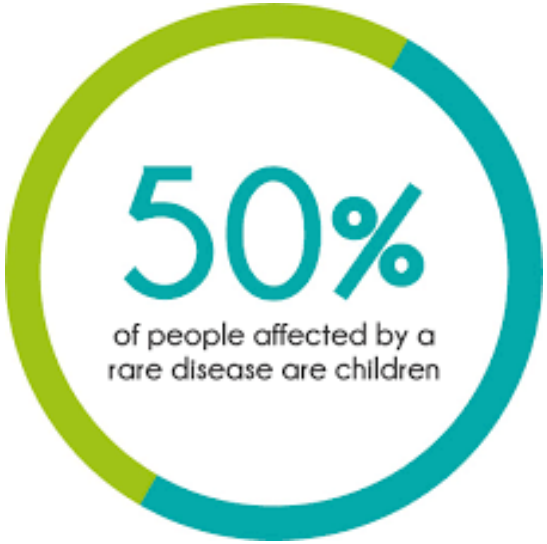
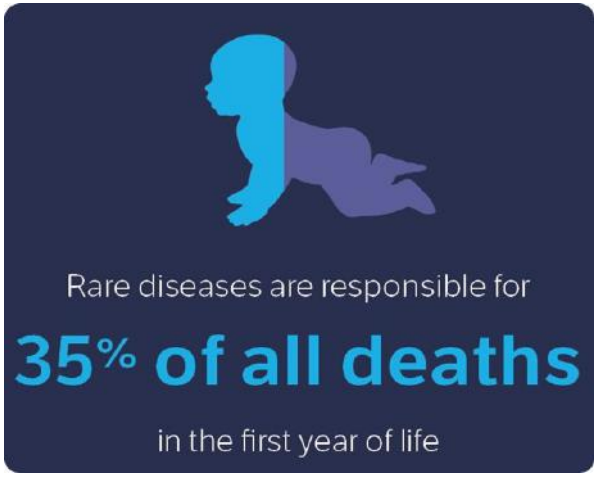
High Unmeet Need is still there !!!





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What Others Think?

COMMISSION REPORT "Still **the use of rewards was limited to 55 % of the completed PIP**s …the **PUMA** concept with its specific reward **has failed to deliver**."

COMMISSION REPORT "Regulation works best in areas where the needs of adult and paediatric patients overlap. Especially, in **diseases that are rare and/or unique to children** and which in many cases are equally supported through the orphan legislation, **major therapeutic advances often failed to materialise** yet.'"

Commissioner for Health and Food Safety, **Vytenis Andriukaitis,**
`"When we consider the advances in adult oncology, it **upsets me deeply** that we have **not made** the same progress in **treating the cancers that affect children**," commented . "In the **next 10 years we must** focus on making similar breakthroughs for children."

The MEP who was rapporteur for the Paediatric Regulation - Françoise Grossetête
"I am **all the more afraid** that the **ongoing incentives review** carried out by the Commission, together with the current **anti-innovation climate**, with particularly **harsh criticisms** against the Orphan Drugs Regulation, **would harm children access to medicines in Europe**,".

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There are Areas of Improvement

Today's challenges can be solved if **all stakeholders fully see and understand their position and their value in the chain** of drug development

- Scientific Level
- Technical Level

The Reality

REGULATORY NEEDS IN PEDIATRIC DEVELOPMENT

- Neonatals
- Pediatric Cancers
- Rare Diseases

REGULATORY SUCCESS IN PEDIATRIC AND ORPHAN DEVELOPMENT ?

Example : Between 2007 and 2015

150 PIPs agreed for medicinal products with ODD

Number of completed PIPs for medicinal products with ODD: 8

Number of authorisations of paediatric indications: 9 (+6 in 2016)

3 orphan medicinal products: Two-year extension of the market exclusivity period

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Scientific Level

- Rethink the existing models, develop non-conventional designs, and innovative methods and tools
- Involve patients and PO early in the research
- Develop Better Protocols
- Develop Biomarkers and their qualification
- Alternatives to animal models , more in silico !
- Patient-relevant outcome measures (PROMs) & RWE
- Use of existing Data Bases and Biobanks
- SHARE, OPEN and FAIRIFY DATA

Technical Level

- **Common guidelines** in development on specific diseases
- **Use New Technologies and IT tools for the evaluation and decision**
- **Patient registries**
- **Disease registries**
- **Natural History Data collection and Data Sharing**
- **Involvement of patient** in the full lifecycle of the product
- **New Statistical tools**
- **More effective reporting AE** system

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WORK ON A NEW DEAL , WE NEED A NEW BLUEPRINT

A new blueprint to fast-track R&D, cut costs and improve access

- **Begin early,** at the very moment when medicines are being researched and developed.
- **Deepen during the process** and include stakeholders farther **downstream**
- **Look beyond innovation in scientific platforms,** products and technologies
- **Look and work for innovation in strategy , policy , access and thinking**

Several tools, techniques or methodologies exist today that can allow medicines to come to market in greater numbers and for lower investments, by accelerating development timeframes but also reducing the costs incurred by companies from early designs to approval.

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But is it feasible ?

This project has received funding from the European Union's H2020 research and innovation programme under grant agreement No 780262

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But is it feasible ?

Share4Rare was built to

- Validate that a secure research platform driven by patients and POs is feasible and sustainable
- Allow patients to drive research in a way that is cheap, fast and of high quality
- Develop a place where patients are the owners and controllers of their data
- **Develop a business model without selling patient data**

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European Commission

Share4Rare

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But is it feasible ?

Where we are now

- Co-created and validated the S4R concept
- Finished the technical design and development
- Launched the first research pilots
- **Passing from the Pilot to the Sustainability phase**
- **Refining our value proposition**
- **Exploring collaboration in order to foster**

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Share4Rare

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To promote patient centric research in the rare and paediatric diseases

- Find ways to build **sustainable** global drug development model together
- Find **innovative processes** in R&D but most importantly we need to find **innovative thinking processes** too
- Work **together** and design a common future
- **And build it on Trust**

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