VERISTAT

FUTURE TRENDS ON EUROPEAN REGULATIONS ON MEDICINES

13 DECEMBER 2022

A LIVE VERISTAT AND TOPRA WEBINAR

VERISTAT SPEAKERS

Xavier Luria, MD
Vice President,
Regulatory Affairs Europe,
Veristat

Michael Paul
Associate Director,
European Regulatory Affairs,
Veristat

Paula Meler
Associate Manager,
European Regulatory Affairs,
Veristat

To contact Veristat directly after the webinar, please email us at **insights@veristat.com**

MEET VERISTAT

Regulatory, Clinical Development, and Commercialization Experts

At Veristat, we are problem-solvers.

Scientific experts.

Regulatory upholders.

Our teams are passionate and committed to guiding you through a successful clinical trial and regulatory submission process. Delivering regulatory insights to help you bring therapies to market to improve lives.

VERISTAT GLOBAL REGULATORY SOLUTIONS

Trust Veristat's global regulatory experts - expertise with **FDA, Health Canada, MHRA, EMA, EU National Agencies** – and a thorough understanding of the regulatory viewpoints and requirements in each region

FOR DRUGS/BIOLOGICS

Regulatory Strategies/Planning

- Road Map
- Gap Analysis
- Regulatory Consulting Support

Regulatory Operations

- Special Designations/Applications
- Clinical Trials Applications- CTAs/INDs
- Marketing Applications (NDA, BLA, MAA, etc.)
- Agency Meetings, Briefing Meetings, and Scientific Advice (SA) Meetings
- Regulatory Publishing

FOR MEDICAL DEVICES AND IVDS

Regulatory Strategies/Planning

- Road Map
- Gap Analysis
- Regulatory Consulting Support

Regulatory Operations

- Classification Analysis
- Technical Documentation
- Clinical Evaluation Report
- Clinical Investigation
- Performance Evaluation Report
- Quality-related Processes

EU Specific Operations
Transition to 2017/745 (MDR)
Transition to 2017/746 (IVDR)

AGENDA

- European Pharmaceutical and Medtech Industry
- New Pharmaceutical Strategy for Europe
  - Introduction
  - Unmet medical need
  - Accessibility and affordability
  - Competitive and innovative European pharma industry
  - Ensure EU voice globally
  - Enhancing resilience – diversified and secure supply chains, environmental sustainable.
- Conclusions/final remarks
- Veristat Capabilities

EUROPEAN PHARMACEUTICAL AND MEDTECH INDUSTRY

Current pharma legislation from 2005 and med-pharma from the 90s

Recollection of experience:

- Results from ongoing regulatory pathways: orphan, pediatrics, conditional approval, etc.
- COVID-19
- Antibiotics resistance
- Reimbursement issues

New Regulation for MD in 2021 and for IVD in 2022

Several changes in terms of requirements and all MD and IVD should be renewed according to the new regulations within the next few coming years

NEW PHARMACEUTICAL STRATEGY FOR EUROPE

Adoption

› 25 November 2020 by the European Commission

Objectives

› Fulfilling **unmet medical needs**
› Ensure **accessibility** and **affordability** of medicines
› Supporting a **competitive** and **innovative** European Pharmaceutical Industry
› Diversified and **secure** supply chains, **environmentally** sustainable
› Ensure a **strong** EU voice

Type of Act

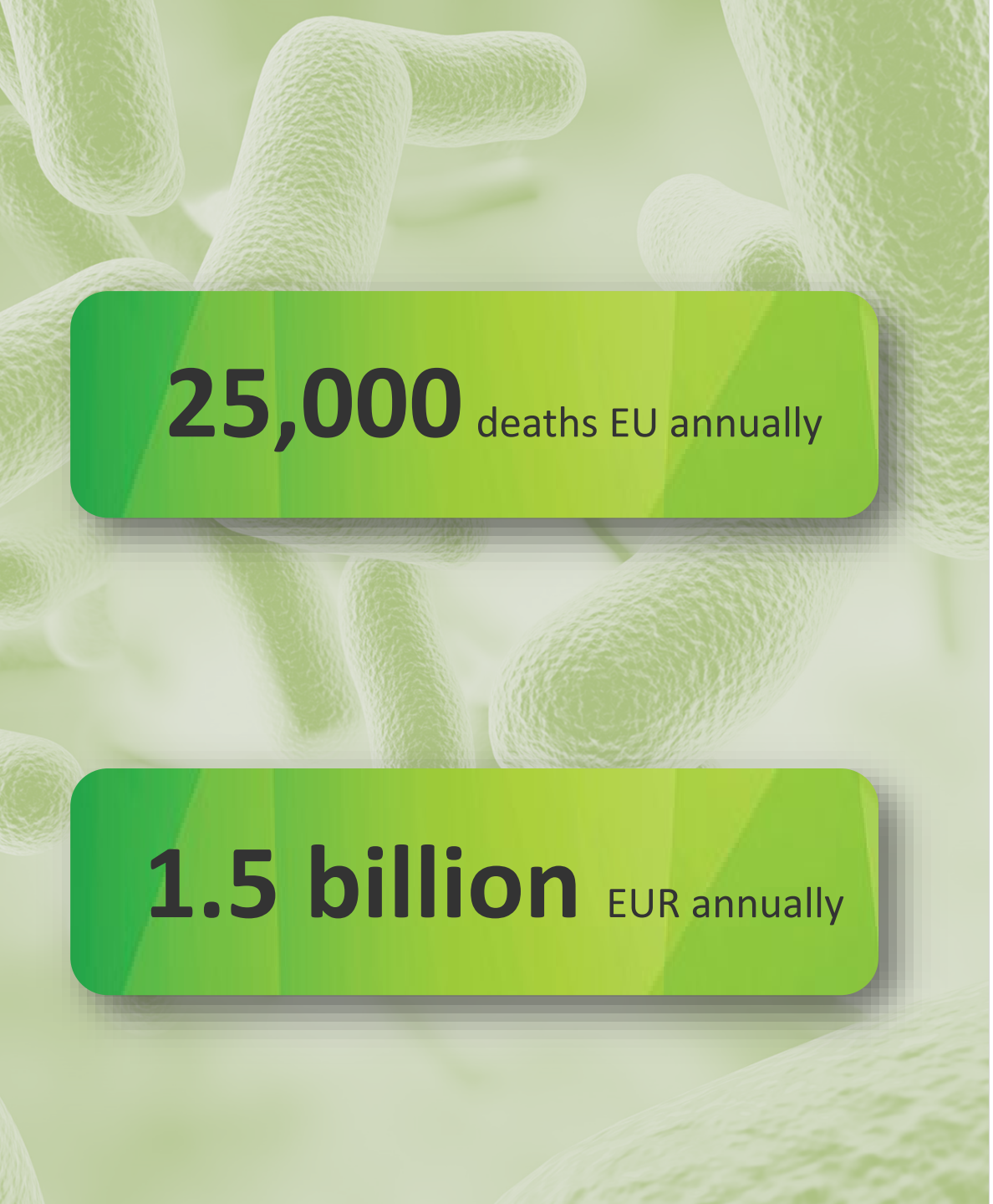
› Proposal for a Regulation
› The Commission published its Roadmap on the revision of the general pharmaceutical legislation in 2021.

FULFILLING UNMET MEDICAL NEEDS

How to Define Unmet Medical Need?

A condition for which there exists no satisfactory method of diagnosis, prevention or treatment in the Union or, even if such method exists, in relation to which the medicinal product concerned will be of major therapeutic advantage to those affected (Article 4 paragraph 2 of Commission Regulation EC No. 507/2006).

The New Pharmaceutical Strategy has the aim to **Prioritise unmet medical needs**



ANTIMICROBIALS - AN EXAMPLE OF UNMET MEDICAL NEED

New EU Health Action Plan Against Antimicrobial Resistance

Aim: Delivering innovative, effective and sustainable responses to Antimicrobial Resistance (AMR).

Key Objectives:

- Making the EU a best practice region
- Boosting research, development and innovation
- Shaping the global agenda

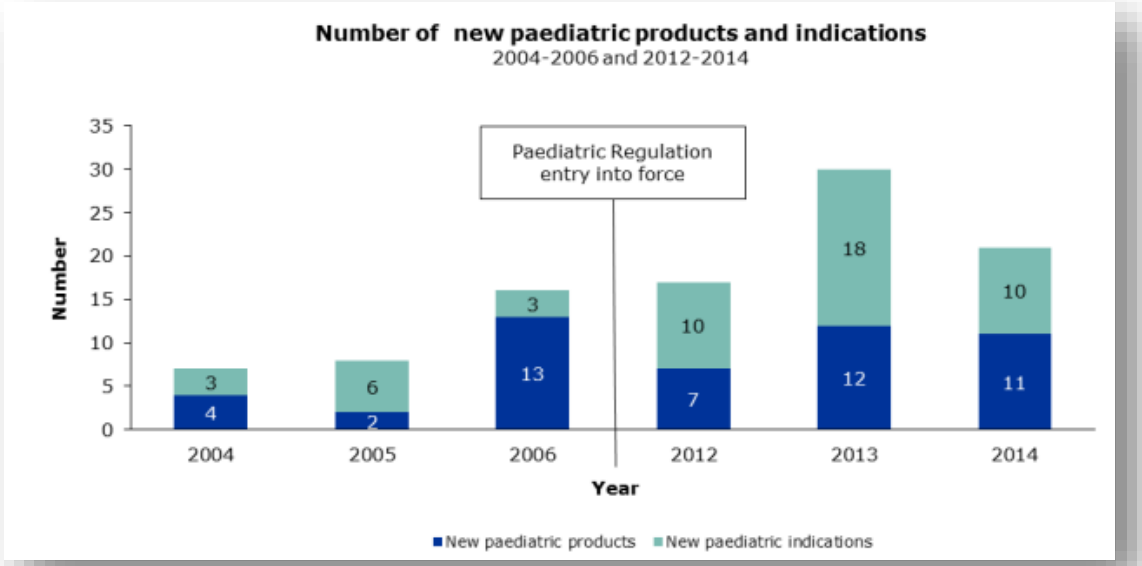
In 2020, **progress report on the AMR Action Plan** was published.

ORPHAN AND PAEDIATRIC LEGISLATION REVIEW

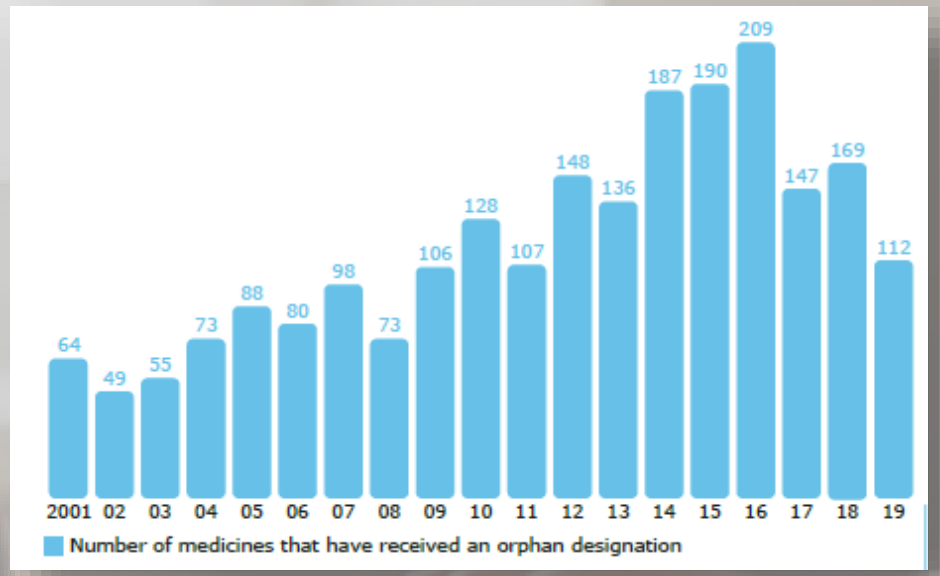
Orphan Regulation (EC) No 141/2000

Paediatric Regulation (EC) No 1901/2006

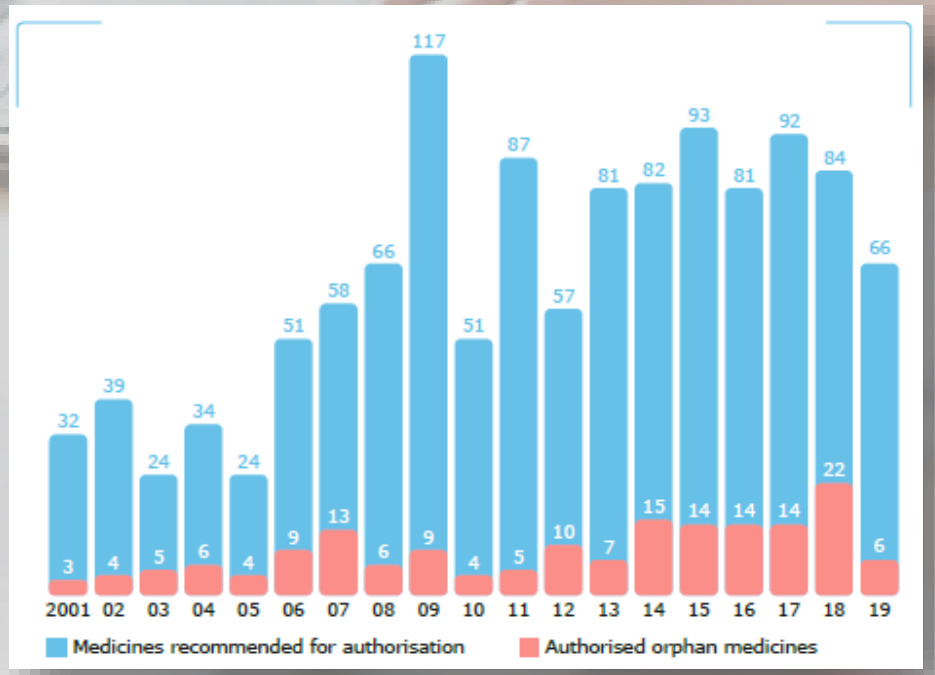
- Impact of the Orphan and Paediatric Regulation
- Assessment of both regulations together
  - Tackle a niche of lack of medicinal products
  - Often address the same therapeutic areas
  - Overlapping incentives



https://health.ec.europa.eu/system/files/2020-06/paediatrics_10_years_ema_technical_report_0.pdf



https://www.ema.europa.eu/en/documents/leaflet/leaflet-orphan-medicines-eu_en.pdf



ORPHAN REGULATION (EC) NO 141/2000

Objectives

- Research and development and placing on the market
- Ensure quality of the treatment

Criteria

- Prevalence OR Insufficient return on investment
- Life-threatening or chronically debilitating condition
- Medical Plausibility
- No satisfactory treatment exists in the EU, or, if it exists, the product in question should provide a significant benefit to patients affected.

Incentives

- Fee reduction.
- Access to centralised procedure.
- At the time of the marketing authorisation – **10 years of market exclusivity.**

ORPHAN REGULATION (EC) NO 141/2000

Main Findings

1. Not sufficient effective to catalyse the clinical development in areas of greatest unmet medical need

95% of rare disease still have no medicines yet been authorised.

28% of **142 authorised orphan medicines**, target disease for which there are no alternative treatments

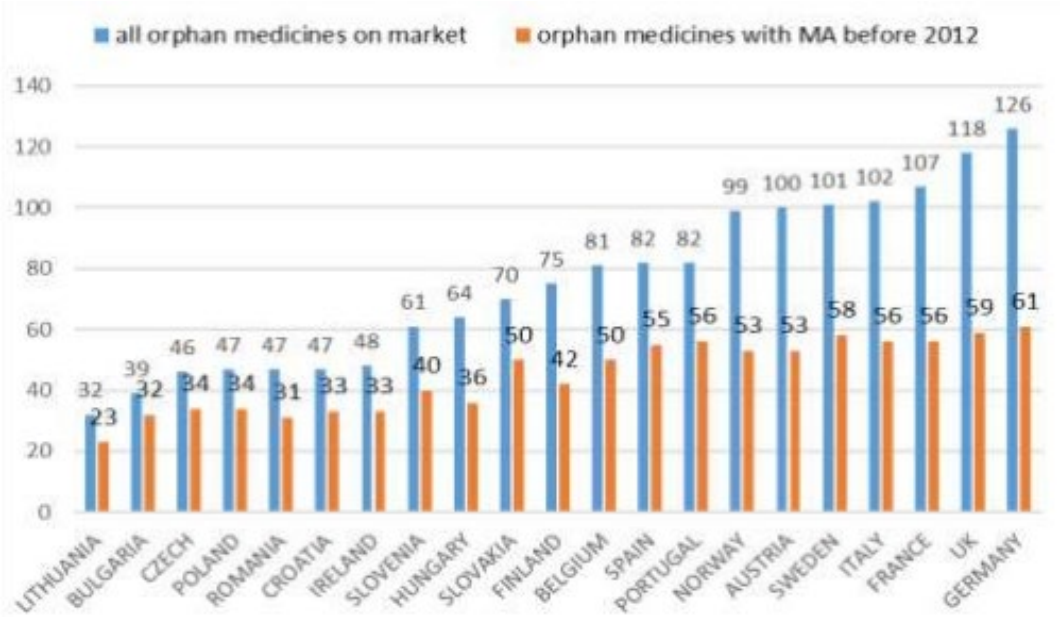
2. Prevalence threshold is questioned

84.5% of analysed rare disease have a very low prevalence (less than 1 in 1.000.000)

Most of the population burden of rare diseases is attributable to the **4.2% diseases** in the most common prevalence range (1–5 per 10,000).

3. Criterion of “Innsufficient return on investment”

4. Accessibility of Orphan Medicinal Products



https://health.ec.europa.eu/system/files/2020-08/orphan-regulation_eval_swd_2020-163_part-1_0.pdf

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PAEDIATRIC REGULATION (EC) NO 1901/2006

Objectives

- High quality clinical research in children
- Ensure that medicines used by children are specifically authorised for such use with age-appropriate form and formulations

Obligations

- Paediatric Investigation Plan (PIP) or a derogation (waiver) should be agreed with the Paediatric Committee (PDCO).
- Compliance is checked at the time of the marketing authorisation application.

Incentives

Incentives *if PIP is completed and agreed studies conducted:*

- **Six-month extension** of supplementary protection certificate (SPC).
- **Two-years extension of the orphan market exclusivity** for orphan medicines.

PAEDIATRIC REGULATION (EC) NO 1901/2006

Main Findings

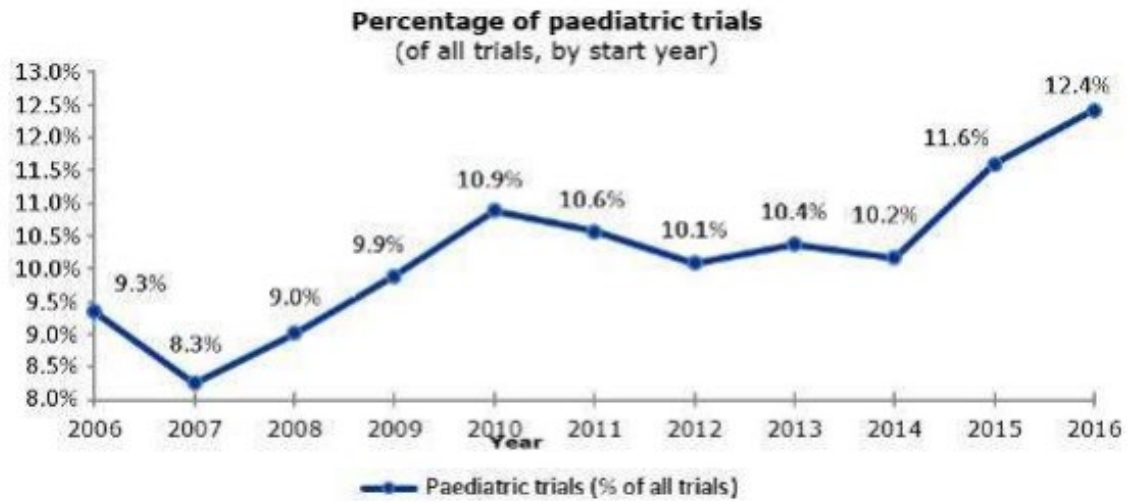
1. Increase clinical research involving children, most of cases linked to adult development.
2. Extensive use of waivers

500 Waivers

1000 PIPs

3. Deferral
4. Not effective in boosting the development of innovative medicines for children with rare diseases.
5. Little use of rewards
   - Paediatric Use Marketing Authorisation (PUMA).

Only 6 Medicines authorized with PUMA, by 2018.



https://www.ema.europa.eu/en/documents/leaflet/leaflet-orphan-medicines-eu_en.pdf

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ORPHAN AND PAEDIATRIC LEGISLATION REVIEW

Medicinal Product for Rare Disease

- Variable duration of the market exclusivity period.
- Changes in the current criteria for ODD.
- Novel incentives for products addressing unmet need in rare diseases and rare paediatric disease.

Medicinal Product for Paediatric Disease

- Link the 6-month SPC extension reward to place the product in most/all Member States, OR to medicines addressing unmet medical need.
- Further explore PUMA scheme
- Novel incentives for products addressing unmet medical need

Modification to the current system of reward, different options are considered.

ENSURE ACCESSIBILITY & AFFORDABILITY OF MEDICINES

Accessibility

- Not all authorised medicinal products marketed in all EU countries.
- The commission will review the system of incentives
- Generic and Biosimilar medicinal Products
  - Provides accessible and affordable medicinal products.
  - The Commission is considering targeted policies to support generic and biosimilar competition.

Affordability

- Revising the pharmaceutical legislation to make it more conducive to competition and reinforce affordability in the EU pharmaceuticals market – 2022
- Cooperating with the relevant national authorities within a group of competent authorities on pricing and reimbursement and payers to exchange information on sustainable health systems, pricing, cost-effectiveness, payment, procurement policies and affordability - including of cancer treatment - 2021-2024

INNOVATION AND DIGITAL TRANSFORMATION

Competitive and Innovative European Pharmaceutical Industry

COVAX

CEPI Gavi unicef World Health Organization

Vaccine platform to monitor the effectiveness and safety of vaccines, supported by an EU-wide clinical trials network.

1+ Million Genomes Initiative

Personalised medicines and gene and cell therapies

1+MG

EXSCALATE 4COV

Excalate4COV

Computing and artificial intelligence

https://www.gavi.org/sites/default/files/covid/covax/COVAX-Supply-Forecast.pdf
https://www.exscalate4cov.eu
https://digital-strategy.ec.europa.eu/en/policies/1-million-genomes

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INNOVATION AND DIGITAL TRANSFORMATION

- **Combination products, including Medical Devices**
  - New Medical Device and In-vitro Diagnostics Regulations (EU 2017/745 MD & 2017/746 IVD)
- **Repurpose off-patent medicines for new therapeutic uses**
  - EMA and Heads of Medicines Agencies (HMA) launched a Pilot project to support repurposing of medicines of not-for-profit organisations and academia.
- New methods of evidence generation and assessment – **Real World Evidence/Data**
  - OPTIMAL Framework – 3 pillars Operational, Technical & Methodological
  - Reference article - Real-World Evidence in EU Medicines Regulation: Enabling Use and Establishing Value. Peter Arlett, Jesper Kjær, Karl Broich, Emer Cooke. Clin. Pharmacol. Ther. , 111 (1)1: 21-23 (2022)

https://www.ema.europa.eu/en/news/repurposing-authorised-medicines-pilot-support-not-profit-organisations-academia

REAL WORLD DATA

EMA: Workplan with 11 workstreams

1. Darwin EU
2. Data Quality
3. Data Discoverability
4. Skills
5. Business Review
6. Analytics Capability
7. Expert Advice
8. Data Governance
9. International Collaboration
10. Stakeholder Engagement
11. Veterinary Data Strategy

NEW PHARMACEUTICAL STRATEGY FOR EUROPE – LOOKING FORWARD

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‘EUROPEAN ONE VOICE’

A vision for a stronger European Regulatory landscape

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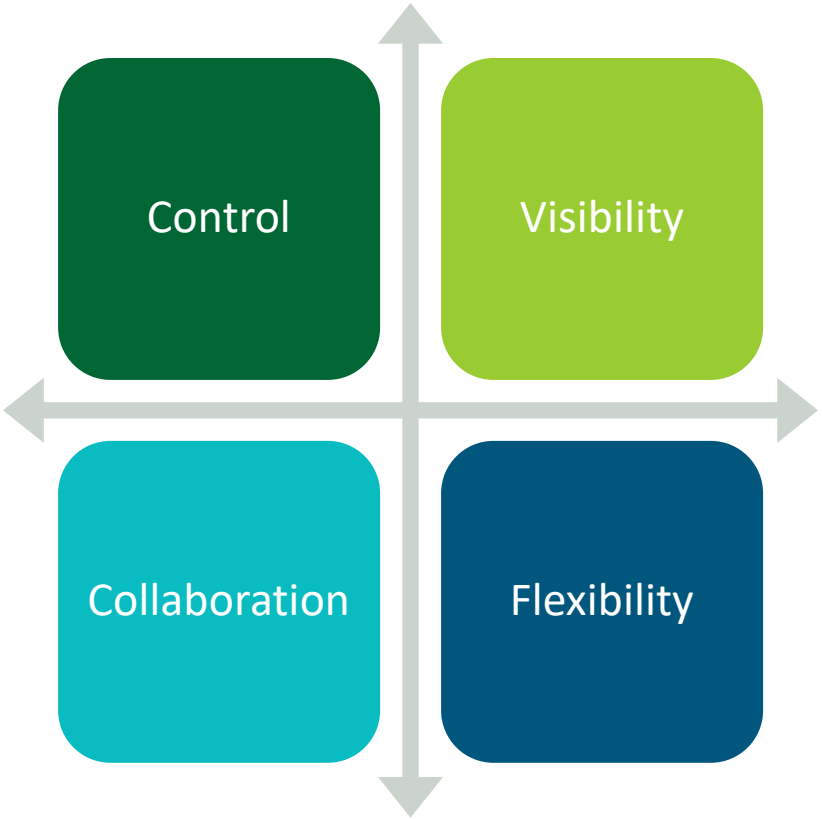
ENHANCING RESILIENCE

4 Elements of a Strong and Resilient Supply Chain:



This can be difficult to achieve but it is possible within the pharmaceutical industry.

Key Aspects of Strong Resilient Organisation:

- Strong foundation & Value
  - Organisational Structuring and securing the right leadership
  - Strategic planning – strategic milestone at the right time
  - Manage supply and demand appropriately
- Lesson learned from Pandemic
  - Timeframes
  - Workload to expedite development of vaccines
  - Initiating clinical trails - Introducing Real World Data (RWD) and Real World Evidence (RWE) into clinical development and regulatory decision making;
- Intellectual property
  - incentives and rewards are the foundation on which innovation is built – Patent, orphan and Peadatric incentives etc

CONCLUSIONS
&
FINAL REMARKS

QUESTIONS

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THINK BOLD. THINK SCIENTIFIC. THINK VERISTAT.