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SCIENCE MEDICINES HEALTH

Developing a framework of collaboration between EMA and Academia

Partnership with academia: today and tomorrow

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Presented by Marisa Papaluca on 15 June 2016
Senior Scientific Advisor – European Medicines Agency R&D support Division

Collaborative frameworks

EU Medicines Agencies Network Strategy to 2020

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- Support for *patient focused innovation* and contribute to a *vibrant life science* sector in Europe
  - Facilitate innovation to ensure patient access to new medicines
  - Greater collaboration across network to support innovation

- Consider further incentives to *support beneficial innovation*, including a European early stage innovative medicines designation, with subsequent *optimisation of development*

- Ensure timely access to new beneficial and safe medicines for patients
  - Better understanding of existing tools (conditional MA, accelerated assessment…) and *prospective planning* of their use

Origins of new medicines [*EU 2010-2012*]

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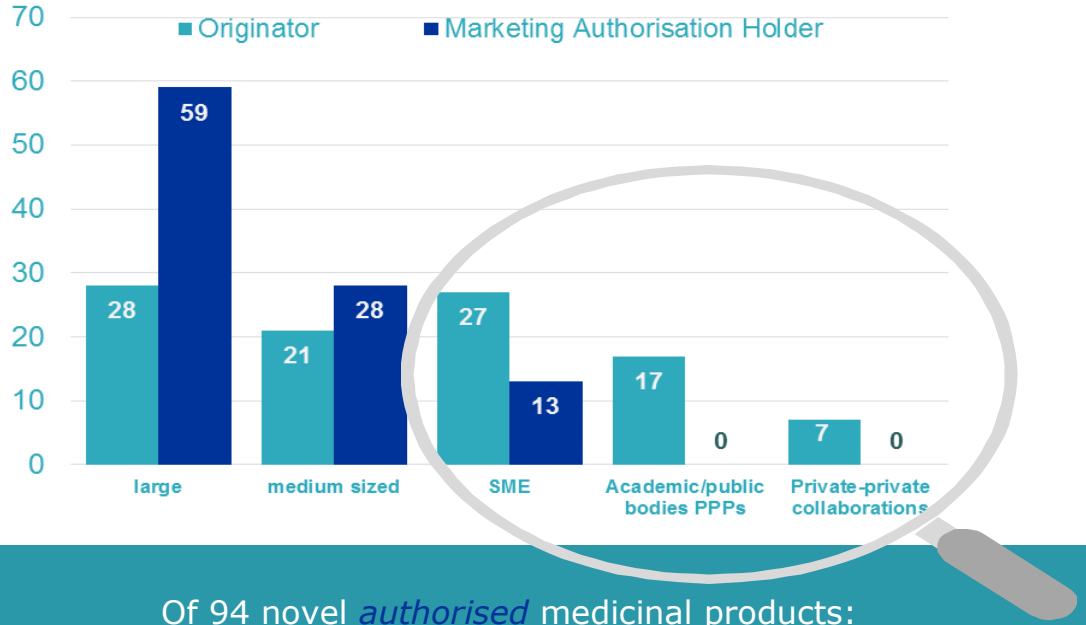

Of 94 novel *authorised* medicinal products:

- Large majority marketed by large or intermediate sized companies.
- SMEs and academia at the origin of innovation.

EMA and Academia collaboration today

(non-exhaustive snapshot)

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Education and communication

- HCP and **Joint HCP and PO working party**
- Participation to **education programs** from both sides e.g. Academic Master courses, EMA webinars
- **PPP educational programs** (e.g. IMI Pharmatrain, Safescimet, Emtrain)
- Participation to **scientific events**, workshops and conferences (e.g. Joint workshops on specific themes, conferences addressed to HCP, Academia, patients and society)
- **Joint publications** in peer reviewed journals

Research

- **Networks Platforms** for advancing good practices in research and share experience (ENPREMA, ENCEPP) including regulatory science
- **Regulatory outcomes research** (e.g. Experts detached at the Agency)
- **Commissioned studies** (e.g. in PharmacoVigilance)
- **Cooperation in EU research projects** (e.g. IMI PROTECT, WEB-RADAR, Adapt Smart)

Medicines development and evaluation

- **Product Specific support**
  - Provide R&D advice and MA input in decision-making: membership in Scientific Advice, SAGs, Committees
  - Evidence generation (e.g. Registries, Pharmacoepidemiology studies, PG studies)
- **Regulatory science standards setting**
  - Novel methods and biomarkers Scientific Advice and qualification: generating knowledge that supports biological rationale and methods for drug development
  - Participation in specialised expert groups and working parties (e.g. SAWP, AMR, PG, M&S, Stats methods etc)
  - Consultation and dissemination of guidelines

How to develop a drug?

Which **tools** have face validity and are *validated to correlate with a clinical outcome*? (e.g. in a neuromuscular disease)

Valid biomarkers and scales (widely used does not mean validated..)

- Prognostic biomarkers (asymptomatic or early stage)
- Biomarker trajectory, disease activity and severity
- Biomarkers for prediction of response
- Confounding effects must be explored

- Muscle mass (DXA, MRI, CT…)
- Muscle strength (Quantitative MS)
- Performance based measures (6MWT, SPPB…)
- PRO tools (PF-10, AM-PAC, IBM-FRS…): do we have validated scales?
- Intervention are an adjunct to life style measure? Exercise? diet?
- Reversal of lab data relates to improved clinical outcome?

How to develop a drug?

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- **How do I measure a clinically meaningful effect?**
  - Does strength increase lead to functional improvement?
  - Does an score improvement improve clinical outcomes?
  - minimum clinically important benefit?

What endpoints matter?

- **Performance measures+ PRO as co-primary endpoint?**

How do I gather the data and the information?

EMA and Academia collaboration: contribute transforming the R&D

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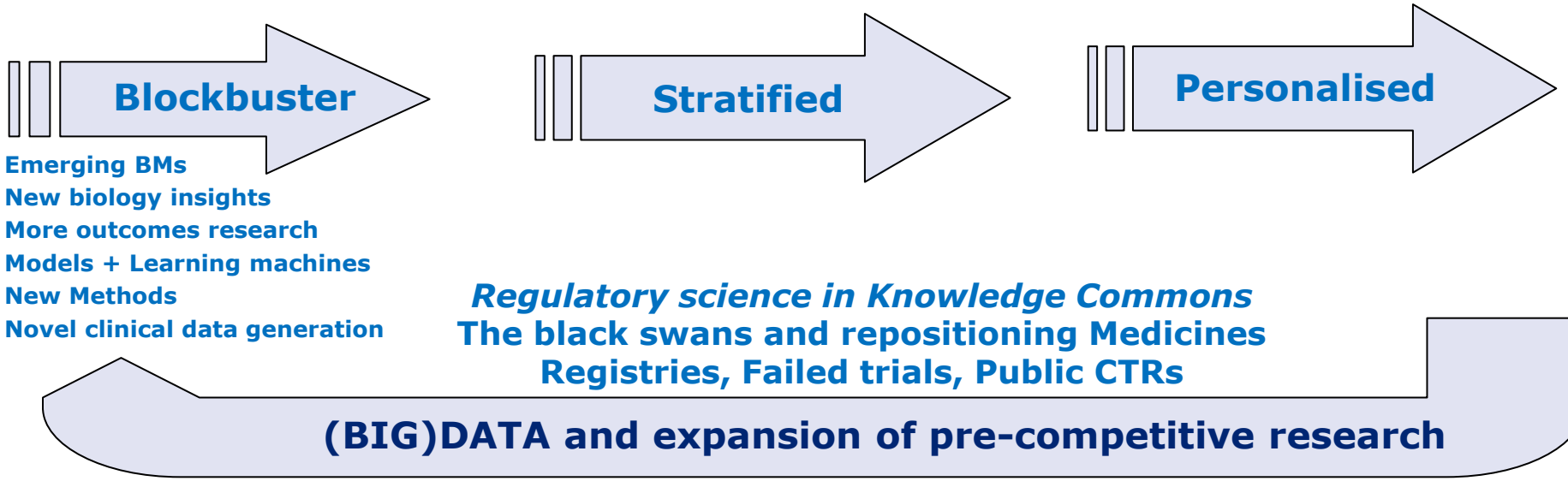

Trans-disciplinary Research

Seamless transition to SMEs, Pharma, Institutions

Public Health policies

Building a bridge to future

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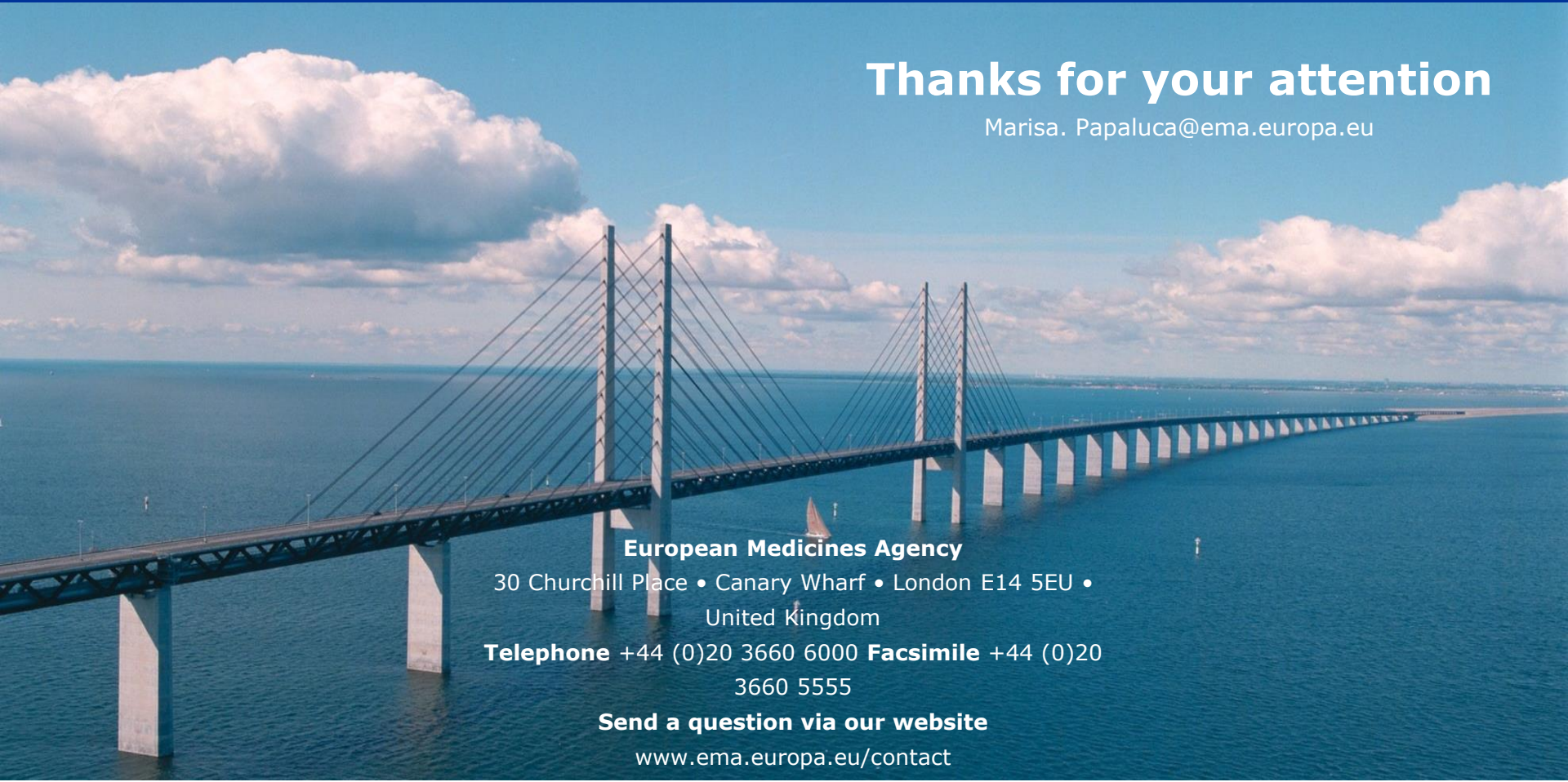

Thanks for your attention

Marisa. Papaluca@ema.europa.eu

European Medicines Agency

30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom

Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555

Send a question via our website

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