Revision of the legislations on medicines for children and rare diseases – Pharmaceutical Strategy

Enpr-EMA meeting

28 Seprember 2021

Fabio D'Atri

DG SANTE

Evaluation of the Regulations for rare diseases and medicines for children

- Strengths and weaknesses legislation 2000-2017 (medicines for rare diseases) and 2007-2017 (medicines for children);
- Commission Staff Working Document published in August 2020;
- Overall the 2 legislations have been successfull, but….

Summary of problems found in evaluation

- Insufficient development in areas of greatest unmet medical needs
  - Developments do not address highest needs for children
  - 95 % rare diseases no treatment option
  - ‘One-size-fits-all’ incentives and rewards <-> unmet needs
- Scientific and technological developments cannot be fully exploited
  - Exclusion from obligation to conduct PIPs (mechanism of action)
  - Innovative CT designs
  - Instruments not adequate for advances in science: biomarkers and personalised medicine
- Availability and accessibility varies across MS
  - Limited link between incentive and placing on market
  - Limited generic competition after expiry of exclusivity periods
- Certain procedures inefficient and burdensome

Objectives of the revision

- To foster research and development of medicines for paediatric diseases in areas of unmet need and in better alignment with patient needs;
- To ensure availability and timely access of patients to paediatric medicines;
- To ensure legislation to be fit to embrace technological and scientific advances;
- To provide effective and efficient procedures, for assessment and authorisation.

Impact assessment - Baseline

- The current Regulation will continue to apply.
- Non-legislative actions developed in the framework of the joint EMA - European Commission Paediatric Action Plan may allow for short term solutions for example in PIP procedures.
- SPC inefficiencies – SPC legislation revision.

Options common elements

- Better support developments in areas of high paediatric unmet needs;
- Strengthen availability of paediatric product in all EU;
- Rethink the PIP procedure for certain products (evolutionary PIP, simplified PIP);
- Take into account the MoA;
- Deferrals.

Options

- Option1
  - SPC extension
  - Rethink other rewards

Options

- Option 2
  - SPC extension only for products addressing UMN

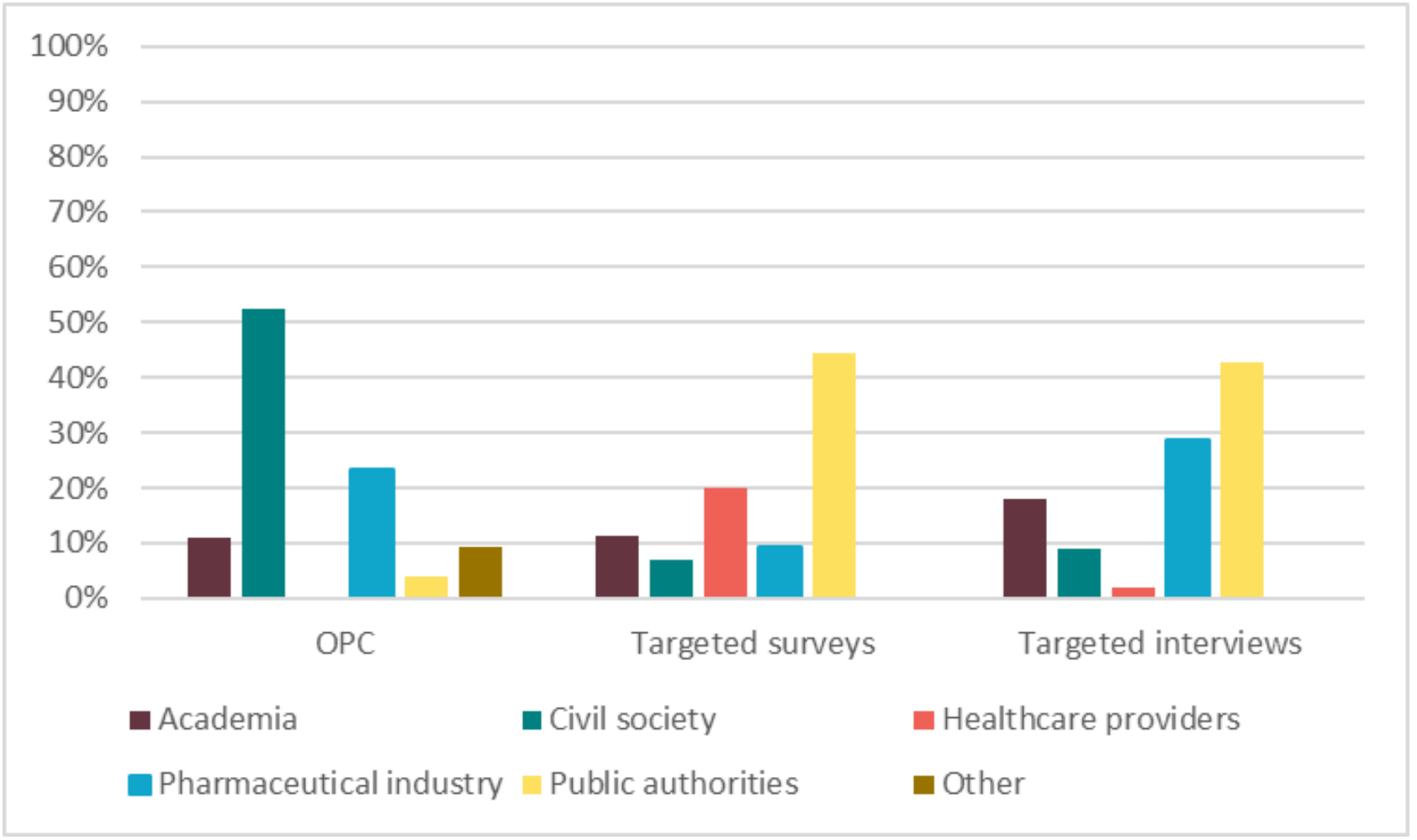
Options

- Option 3
  - SPC
  - Novel reward for products addressing high UMN
- Option 4
  - Novel reward for products addressing high UMN

State of Play

- *Open* Public Consultation (ended on 30 July)
- *Targeted* surveys (options for revision/questionnaires costs of development; interviews with stakeholders)

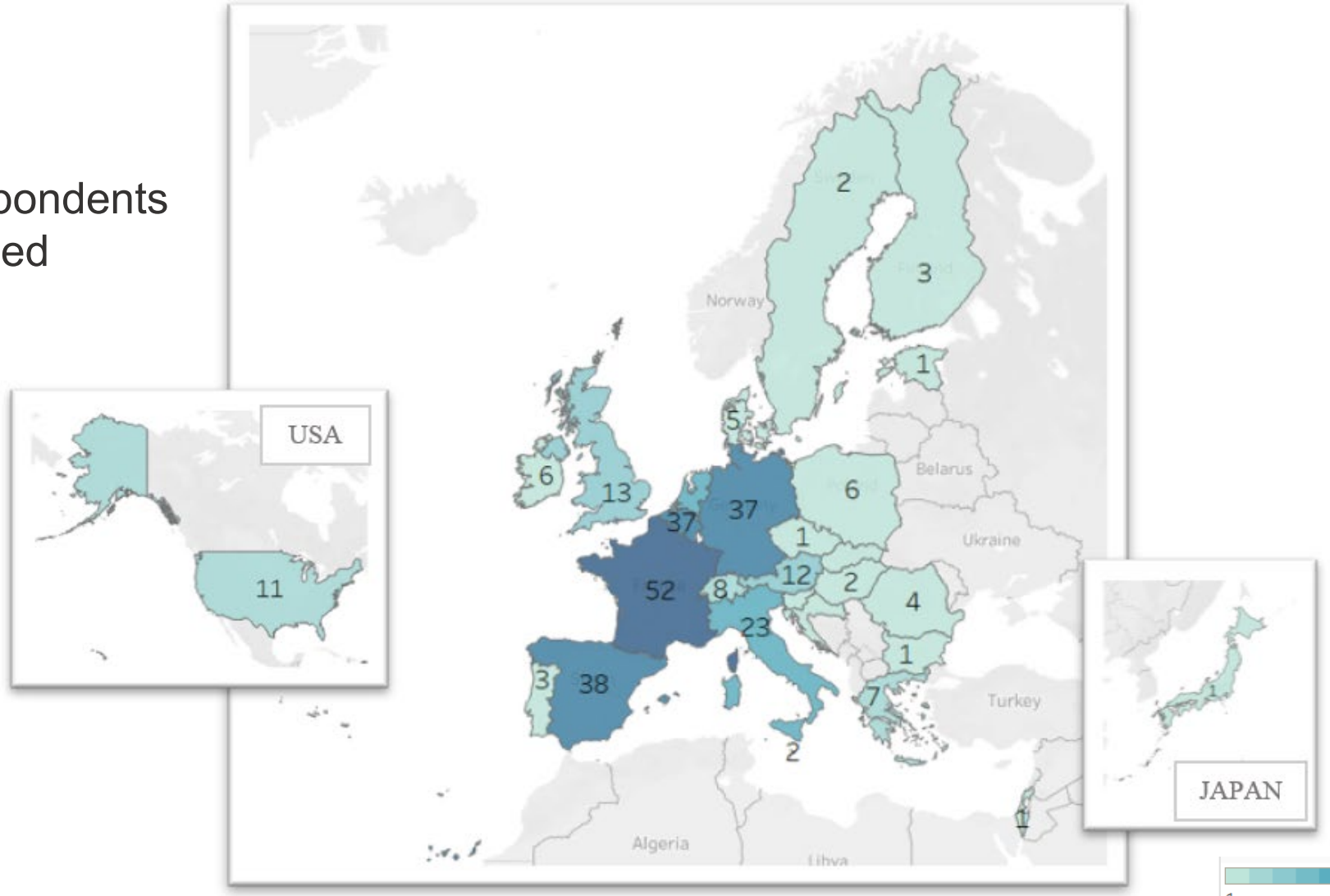
Summary of Responses per Consultation Method



European Commission

Geographic Distribution of OPC Respondents

51% of respondents are EU-based



State of Play

- *Open* Public Consultation (ended on 30 July)
- *Targeted* surveys (options for revision/questionnaires costs of development; interviews with stakeholders)

- Proposal for revision of legislation: 4th quarter 2022

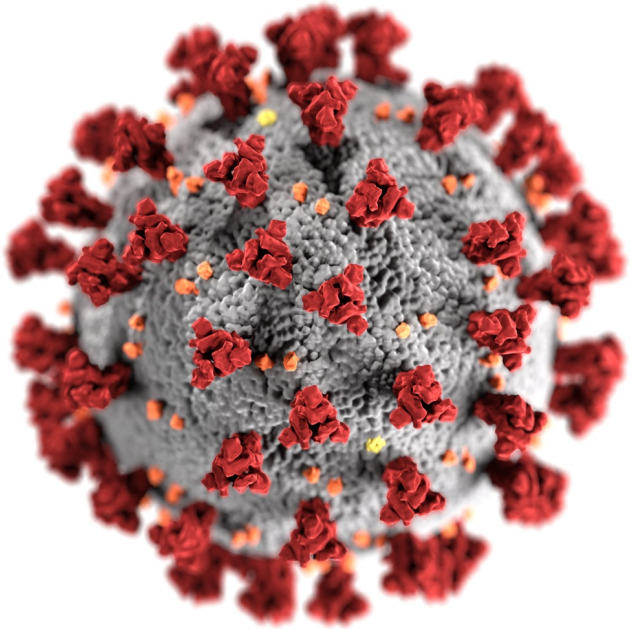
Pharmaceutical Strategy for Europe

- Adopted in November 2020
- Ambitious long-term agenda in the field of pharmaceutical policy
- Objective: creating a future proof regulatory framework and at supporting industry in promoting research and technologies that actually reach patients in order to fulfil their therapeutic needs

European Commission

Aspects highlighted by COVID-19

Crisis management and preparedness



International aspects

Dependency on APIs

Innovation

Shortages

European Commission

A holistic approach covering the full lifecycle of medicines

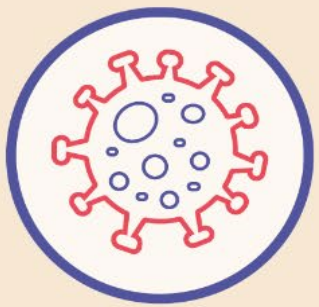
- Research & Development
- Innovation
- Clinical Trials
- Digital & data
- Advanced therapies
- IP/incentives
- Pharma legislation
- Health technology assessment
- …

- Market function
- Procurement
- Manufacturing
- Generics, biosimilars, APIs
- Supply chains
- Environment
- Competition policy
- Trade
- …

Pharmaceutical Strategy in context

European Commission

PHARMACEUTICAL STRATEGY FOR EUROPE



Learning from COVID-19, towards a crisis-resistant system

Ensuring accessibility and affordability of medicines

Supporting sustainable innovation, emerging science and digitalisation

Reducing medicines shortages and securing strategic autonomy

#EUPharmaStrategy

European Commission

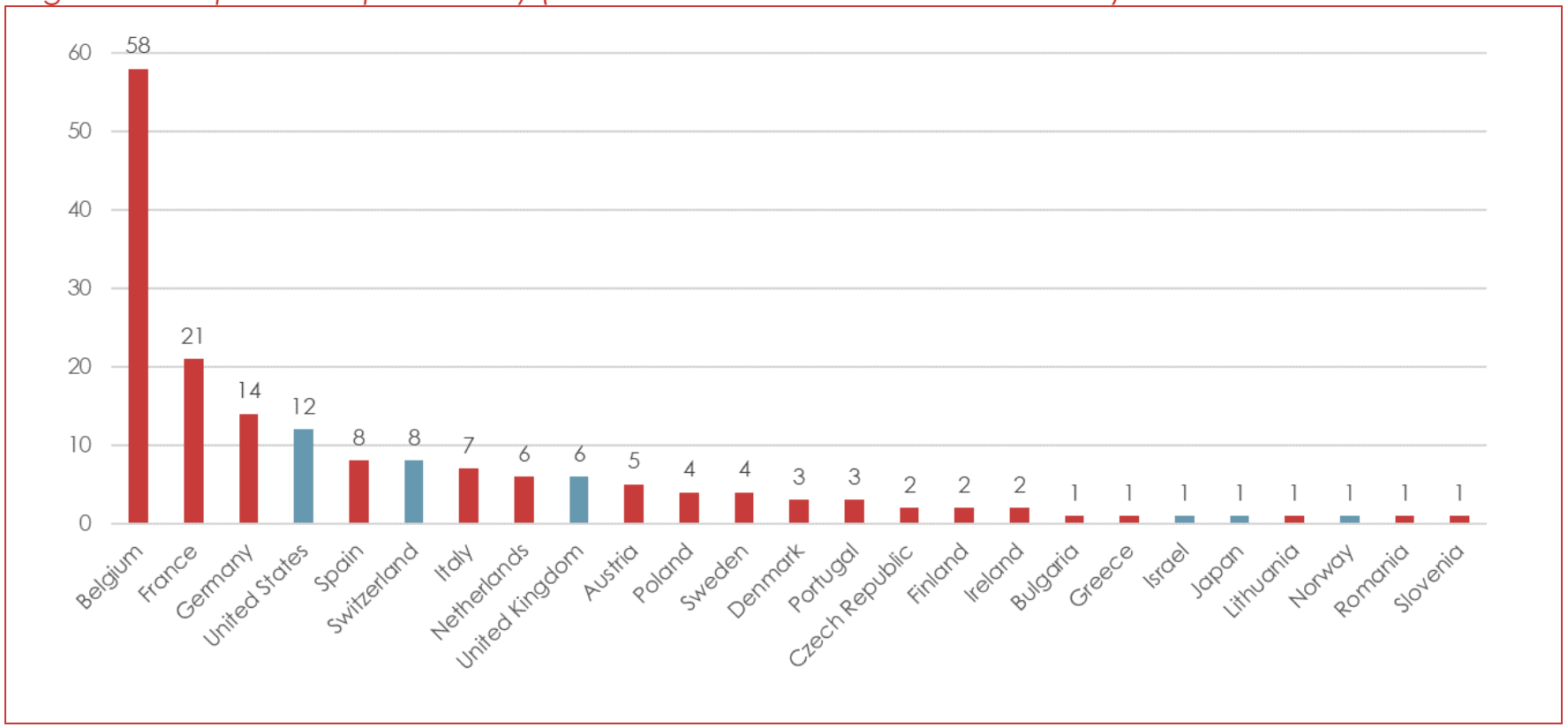
EU Pharmaceutical strategy

The revision of the EU pharma framework will aim at:

- ensuring access to affordable medicines;
- fostering innovation, including in areas of unmet medical need;
- improving security of supply;
- adapting to new scientific and technological developments;
- reducing red tape.

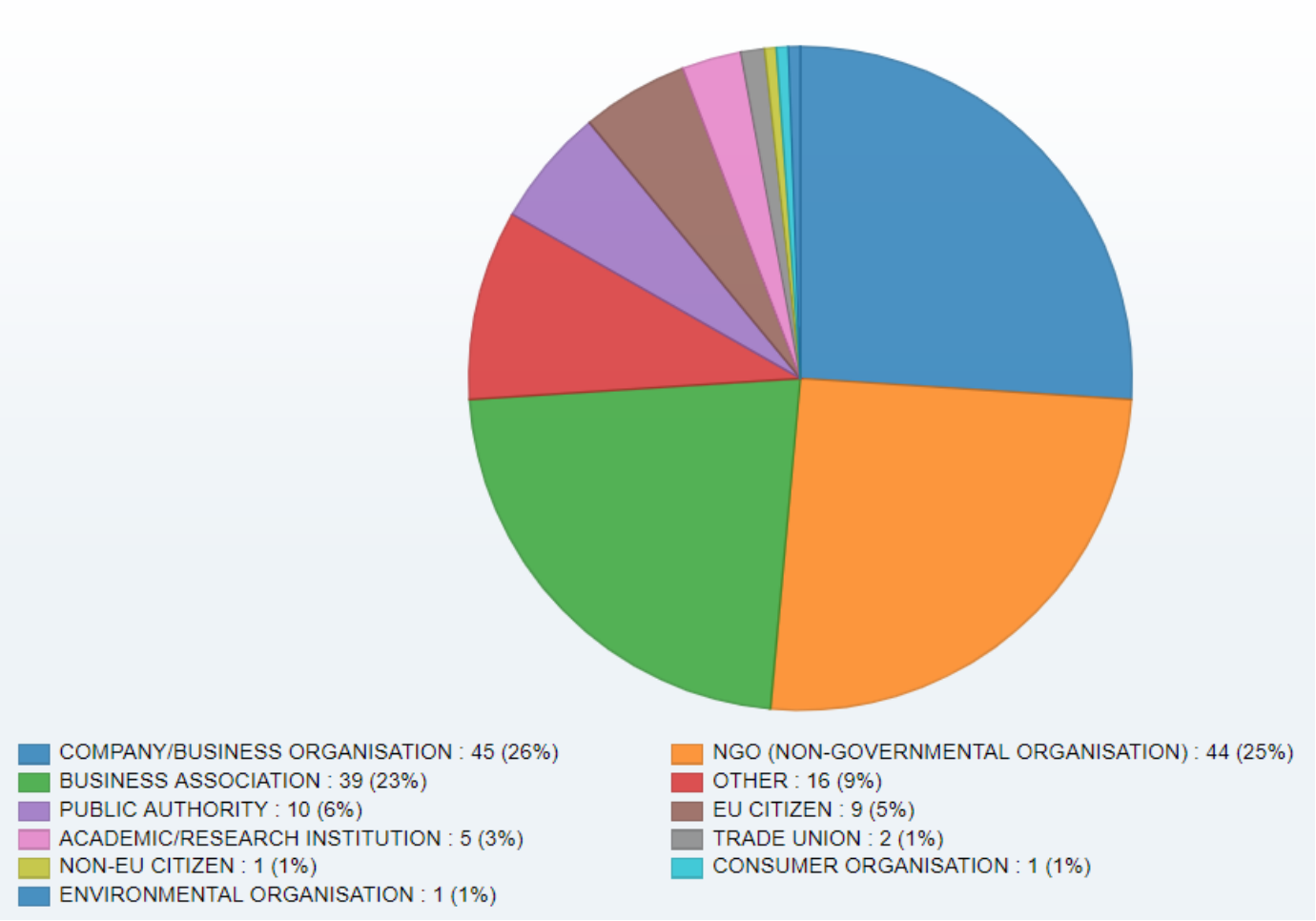
Feedback to the roadmap

*Figure 1 Respondents per country (*non-EU countries are coloured in blue)*



European Commission

Feedback to the roadmap II



European Commission

Feedback to the roadmap

- Evident and strongly positive **support for the revision** of the legislation and a general agreement on the need to make the legislative framework more patient-centred, future-proof and crisis-resistant
- Around 80% of all the stakeholders representing a business association or a company were **concerned about the fitness of the current legislative framework** in terms of its ability to respond to innovations and new developments
- All stakeholder groups emphasised a point raised in the Roadmap about the need to better **conceptualise 'unmet medical need'** (UNM).
- With regards to areas where there is a high unmet need and (perceived) market failure, Antimicrobial Resistance (**AMR**) was mentioned by academic stakeholders, NGOs as well as industry stakeholders.
- **Patient involvement** is stressed by almost all (80%) NGOs as essential at different stages of the revision of the legislation itself, as well as integral component of the new or revised procedures

Consultation activities

- Open public consultation will be launched end of September
  - Evaluation question and forward looking questions
- Targeted consultations will be initiated in parallel with different stakeholder groups

Pharmaceutical Strategy – Forward planning

European Commission

Thank you

European Commission
Public Health information:
http://ec.europa.eu/health/index_en.htm

@EU_Health

https://ec.europa.eu/health/human-use/strategy_en

#EUPharmaStrategy

European Commission