EURORDIS
RARE DISEASES EUROPE

ENSURING ACCESSIBLE, AVAILABLE AND AFFORDABLE TREATMENTS FOR PEOPLE LIVING WITH RARE DISEASES

1st International Conference on Rare Diseases

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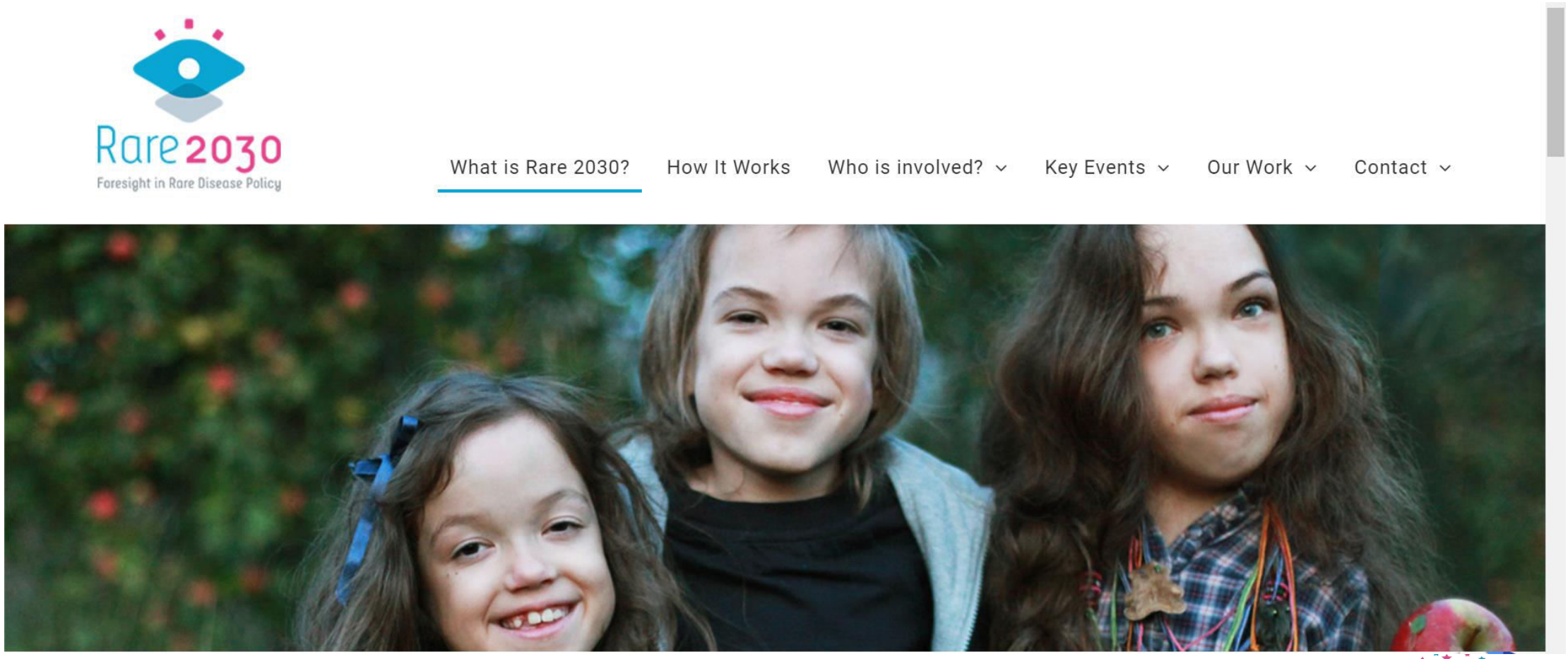
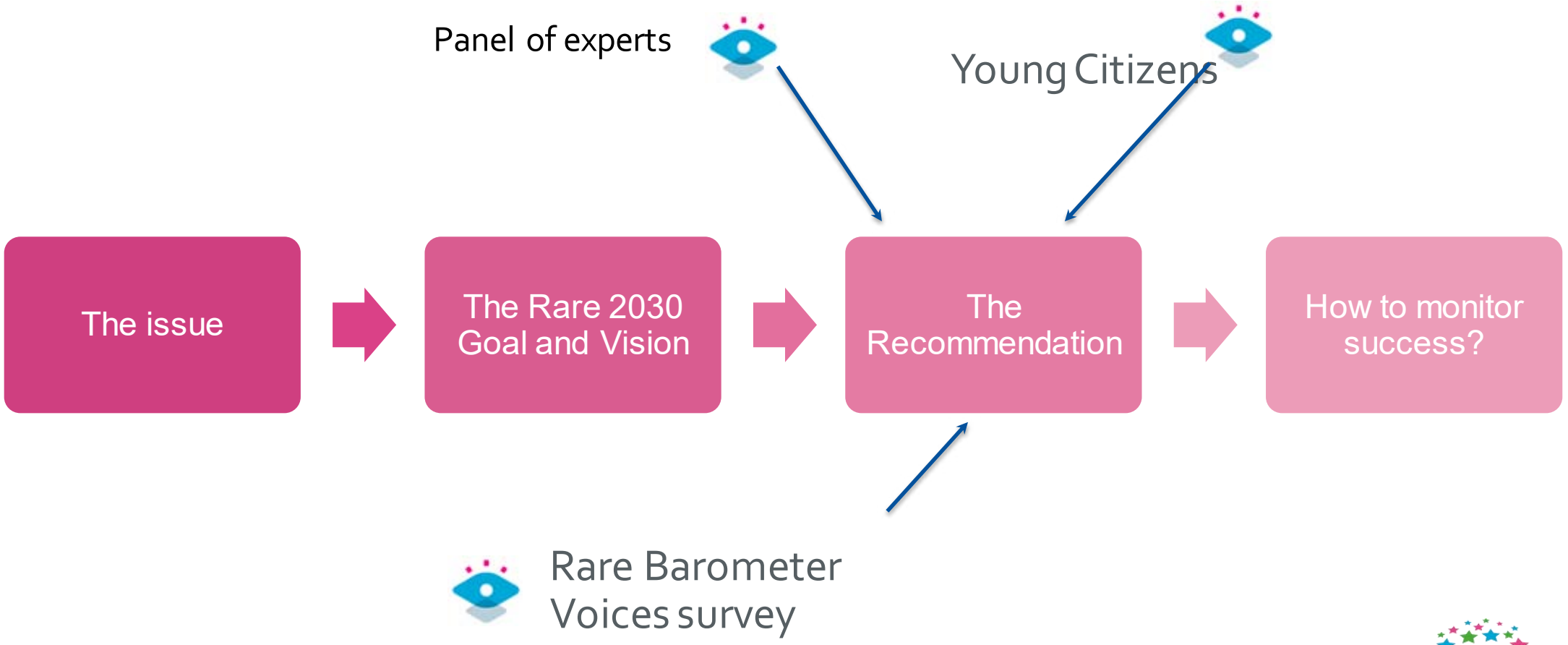
Rare 2030 Foresight Study



More on the recommendation



8 interconnected recommendations

1. *Long-term, integrated European and National Plans and Strategies*

2. *Earlier, Faster and more Accurate Diagnosis*

3. *Access to High Quality Care*

4. *Integrated and Person-Centred Care*

5. *Partnerships with Patients*

6. *Innovative and Needs-Led Research and Development*

7. *Optimising Data for Patient and Societal Benefit*

8. ***Available, Accessible and Affordable Treatments***

What do we recommend to achieve the triple A?

*Establish **streamlined regulatory, pricing and reimbursement policies**. These policies should encourage a continuum of **evidence generation** along the full life cycle of a product or technology as well as the patient journey from diagnosis to treatment access. A European ecosystem able to attract investment in **areas of unmet need, foster innovation, and address the challenges of healthcare system sustainability**.*

Measurable impact

- By 2030

More and better quality curative, stabilising, palliative, assistive, rehabilitative and preventive technologies and therapies available, accessible and affordable

A European competitive ecosystem in the development of RD therapies and a more robust pharma and biotech manufacturing presence

1000 new therapies available

Therapies 3 to 5 times more affordable than current available treatments

The recommendations to achieve the Triple As

- **Streamlined regulatory, pricing and reimbursement** policies
- **Continuum of evidence generation** along the full life cycle
- Early-stage **multi-stakeholder identification of unmet needs** and subsequent priorities and investments
- Functional and efficient EU HTA Framework
- **EU-fund to co-finance the generation of evidence** across EU Member States and reduce uncertainties
- **Adaptive pathways and rapid access mechanisms**
- Both the individual value of products for patients but also the wider societal value
- Developers encouraged to utilise expert rare disease resources and guidance
- **HTA decisions** and reports **at a pan-European level**
- **Post-marketing surveillance** for orphan therapies **at the European level**
- **Efficacy** as well as **safety data** collected from patients **on compassionate use programmes** and pooled at the European
- The role and capacity of **ERNs** in generating, collecting and analysing real world data further defined

On today's panel

- Karolina HANSLIK, Project Senior Manager, EURORDIS
- Angeliki SIAPKARA, Group Manager for MHRA's Benefit Risk Management Group, UK
- Bettina RYLL, MD, PhD Horizon Europe Cancer Mission Board, Melanoma Patient Network Europe, Founder
- Dimitrios ATHANASIOU, EMA Pediatric Committee , EPF, WDO, EAE Board Member