Planning For The Future

Becky Jenner, RSE President

The RSE Board

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Meet the new Board:

- Becky Jenner – President (UK)
- Stella Peckary – Treasurer (Austria)
- Laura Kanapieniene – Secretary (Lithuania)
- Sandrine Eifermann Soutarson – (France)
- Bojana Milanov – (Serbia)





Overall Aims of RSE

- **To represent the interest of people with Rett syndrome and their families, especially in the following areas:**
- **To make Rett syndrome better known to the public, professionals, carers and those who are directly concerned in all European countries.**
- **To improve the communication within the European Rett community.**
- **To promote, as a representative European organisation, the interests of people with Rett syndrome and their families.**
- **To expand RSE to all European countries and to assist, if necessary, in the creation of national associations.**
- **To promote research into Rett syndrome**

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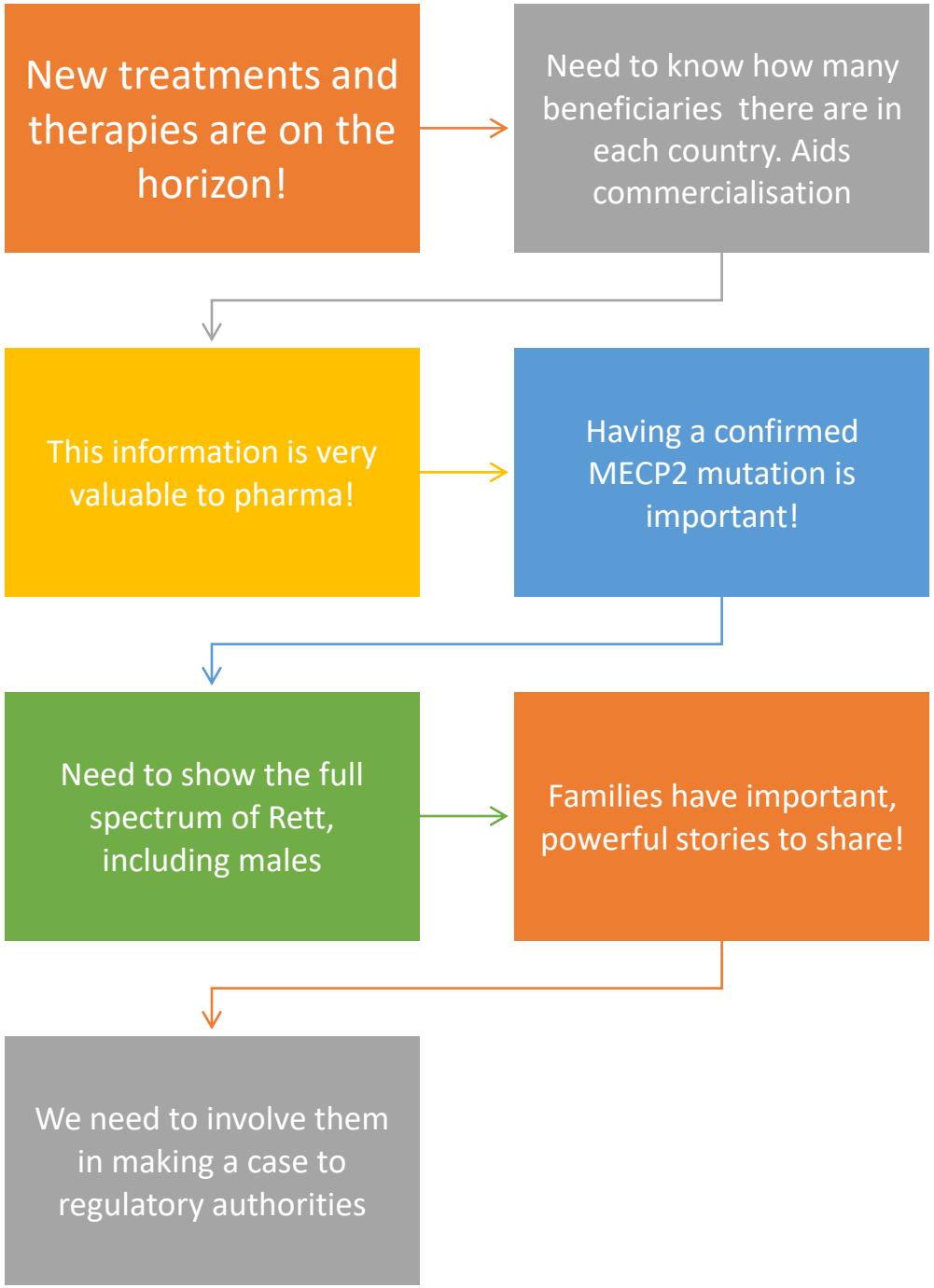
Key Activities Now for Rett Syndrome Europe

- Supporting new or fledging patient organisations to find more families in their countries
- Supporting their engagement and development of the association infrastructure
- Supporting on the development of a patient registry for Europe

Why it is so important now to find families!



RSE - Working with the EMA

They are various ways patients can be involved:

- In early dialogue, Scientific Advisory Groups, public hearings, reviewing documents written by pharma for the community's information
- Task forces and working groups
- As full committee members when it comes to designation of orphan medicines but also with paediatric developments, drug safety committees and advanced therapies
- To provide scientific advice (through the RSE Scientific Advisory Board) and help in designing protocols
- In benefit/risks evaluation (compassionate use, market authorisation, market renewal etc.)
- Find out more from The Patients' and Consumers' Working Party (22 organisations involved) – read full report of how patients have been involved here

Key Tasks For Our Community

To be able to define our unmet needs

Describe what we expect from a new medicine and how we would want it to impact the lives of our children

Be able to explain clearly how we would weigh up the benefits and risks

RSE as an Eligible Organisation with EMA?

Patients' organisations are defined as not-for-profit organisations which are patient focused, and where patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies.

These could be: General umbrella organisations (e.g. representing either European organisations and/or national umbrella organisations), or a European disease specific organisations (i.e. representing national organisations or individual patients on acute and/or chronic diseases).

We then need to show Legitimacy, Mission, Activities, Representation, Structure, Accountability, Transparency particularly around funding – no more than 50% from pharma and more than one pharma company

Stages for the involvement of external experts

- We need to find representatives
- We need to prepare them – training with EMA and/or Eurordis
- We need to make sure they are involved
- They can then expand on the EMA knowledge in a meaningful way
- Their input then needs to be acknowledged.

How does this happen in practice?

- There is a call for information via the EMA database of experts, including for patient representative via eligible organisations i.e. RSE and Eurordis to select the “right” people
- EMA explain the procedures and deal with any administrative tasks e.g. conflicts of interest, confidentiality.
- A round of meetings/questionnaires/interviews/focus groups/patient preferences surveys etc. is initiated
- The results are included in reports and relevant documents.

There should be a continuous loop of feedback, measurement and visibility

To Summarise

The involvement of patient representatives is first and foremost a matter of transparency. It can improve the evaluation of medicines

With good communication and engagement patients and regulators can agree

The EMA is looking to expand use of real-world evidence, patient reported outcomes, patient preferences and patient experience data

To be really effective as patient representatives it is essential that individuals are trained either through the EMA training for external experts, or Eurordis Open Academy

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