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Rare Diseases Europe

Why and How- Involve your organisation with the European Medicines Agency



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Coming to EMEA to give my unique patient perspective!

How do patients participate?

- As experts providing their personal experience with the disease/treatment
- As experts reviewing medical information
- As members (of MB, committees and working parties)
- As observers
- As “facilitator”



Ursula Huffer treating herself for Hereditary Angioedema (HAE



HAE:

causes swellings throughout the body

Often delayed diagnosis, risk of inappropriate treatment.



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First patient to come to EMEA!



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Ursula: Is this easier now?

- transport
- confidentiality
- feed-back



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Retinitis Pigmentosa, a progressive degenerative disease of the retina



Rainald von Gizycki can read white text on black background



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Retinitis Pigmentosa leading to:

- night blindness
- loss of visual field
- tunnel vision and eventually blindness.
- reading ability is affected in the advanced stage of the disease.



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Patient obligations (The checklist)...

- Definition of a patient organisation in EU
- Statutes (rules), registered
- Mission/Objectives (goals to go on EMA:s web-site)
- Activities (be interested in medicines)
- Representativity
- Structure
 - A governing body elected by members
 - Members=patients, carers or representing these



The checklist cont...

- Accountability and Consultation Modalities
 - Whose opinions?
 - flow of information to and from members?
 - Transparency
 - Activities on website
 - Funding on website
 - Conflict of interest
 - Member associations



The patient organisation – the link to patients



- Maria Mavris coordinates EMEA/patient input
 - Difficulties
 - Progress
 - Hopes



Time

- What has been?
- Where are we now?
- Where do you want to be?



Summary:

- Motivation
- Starting the dialogue with EMA
 - The checklist
 - Contact person
 - Mentors
 - Reporting progress





Leaving EMEA after having given my unique patient perspective! Thank you!



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