

Rare2030

Foresight in Rare Disease Policy

GROUP 1



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Young Citizen Conference RECOMMENDATIONS



INTRODUCTION: IF WE HAD A MAGIC WAND

- > We would make sure that every rare disease patient has access to current, clear, and thorough expert information on their disease
- > We would have equal access to healthcare for all patients irrelevant of location or socio-economic status
- > All relevant actors would have the same access to reliable, ethical, patient-driven data



PART | 2

OUR RECOMMENDATIONS

ESTABLISH A EUROPEAN RARE DISEASE AGENCY

> Overarching strategy

- Concentrating expertise, means, and resources
- Create a modular roadmap towards an integrated rare disease approach on EU level

> Focus on key actionable domains

- (Research) funding
- Social support
- Market access
- Data access and management
- Patient registries

RARE DISEASE AGENCY FOCUS AREAS – DIAGNOSIS

> Focus on newborn screening

- Minimum standard of hard to diagnose rare diseases included in newborn screening
- Comparative research on national newborn screening methodologies for best practice identification

> Foster joint-HTA assessment

- EU-wide initiative to assure access and availability of innovative, cost-effective, and safe treatment modalities

RARE DISEASE AGENCY FOCUS AREAS – POLITICAL AND STRATEGIC FRAMEWORK

- > Clarify and reframe the role of ERNs
 - Clarify how individuals can utilize this platform
- > Reframe what it means to live with a rare disease
 - Actively spread awareness to debunk stereotypes and stigmas
- > Stimulate and increase public – private partnerships
 - Fostering PPPs through incentives and other political means

PART | 3

CONCLUSION

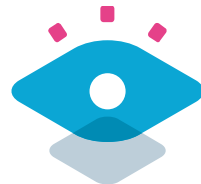
CONCLUSION

- > Focus efforts, expertise, funding through one EU-wide channel: The EU rare disease agency
- > Focus on creating a community of experts, patients, scientists, policy makers that are engaged and willing to contribute
- > Aim for co-creating and full value chain involvement from the ground up
- > Move the knowledge not the patient

THANK YOU FOR YOUR ATTENTION.



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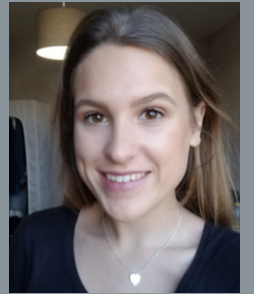
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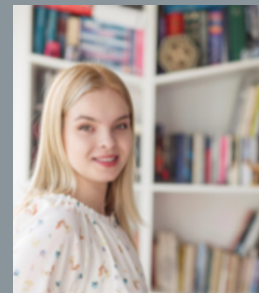
ANA BEGIC



KHADIDJA ABDALLAH



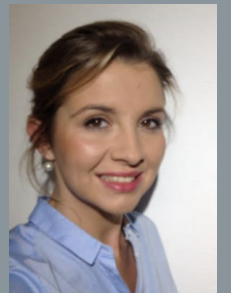
RACHEL CLAYTON



AMINA BERBIĆ



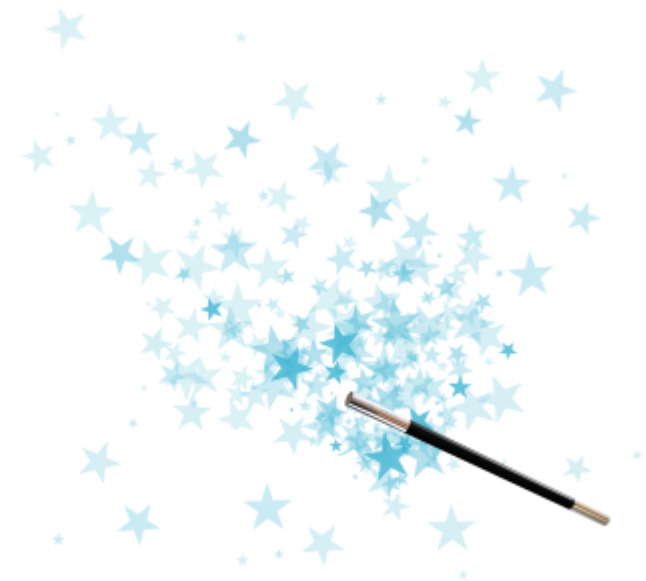
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INTRODUCTION: IF WE HAD A MAGIC WAND WE WOULD ...

- > End discrimination towards people living with a rare disease
- > Give equal opportunities and access to resources for people living with a rare disease
- > Have equal access and availability to medicines and full reimbursement at European level



PART | 2

OUR RECOMMENDATIONS

RECOMMENDATION 1: DEVELOP A MORE COHESIVE AND MORE TRANSPARENT REIMBURSEMENT PROCESS

> Why?

- Individuals do not have a clear understanding of reimbursement procedures as they differ within countries and across Europe
- **Main actors: Insurers (private and public) , pharmaceutical industry, commission on reimbursement, minister of social affairs and health**

> Possible actions

- **Guidelines** created by the EU to encourage standardised processes across member states
- **Put a system in place that works when the state fails at european level –** database/ initiative that monitors patient medicines and treatments + reimbursement

> Potential Challenges

- data protection , transferring processes between localised and standardised , resistance against transparency from industry, patients and insurers (all countries have different systems)

RECOMMENDATION 2: ADDRESS MENTAL HEALTH ISSUES AND SOCIAL CARE CHALLENGES

> Why?

- **Mental health issues often arise with living with rare diseases** and can be overlooked because of prioritising other treatment
- **The quality of life can decrease** if a person lives with mental health issues untreated, might exacerbate symptoms related to rare disease
- Mental health issues are becoming more prevalent in younger societies

> Possible Actions

- Inclusion of mental health aspect in treatment guidelines for rare diseases starting from diagnosis
- Training for mental health specialists on rare diseases

> Potential Challenges

- Not enough specialists, it is not necessarily a priority for doctors, cost

RECOMMENDATION 3: TACKLE DISCRIMINATION VIA EDUCATION

> Why?

- Education on general health is not sufficiently emphasized.

> Possible Actions

- Training on disability and discrimination recommended to educators/ employers. Information more widely available.
- Awareness campaigns to reduce stigmatisation (Rare Disease Day)
- **Level of action:** European / national / regional

> Potential Challenges

- Rare diseases may get lost in the disability community, education may not be sufficient, awareness v action

RECOMMENDATION 4: IMPROVE CROSS-COUNTRY COLLABORATION

> Why?

- **EU countries have different data collection standards**
- **Different care systems** with varying processes across borders

> Possible actions

- **Access to care abroad if needed** – a system at european level that pays health care professionals for the time they spend with **foreign** patients
- **Increasing the number of centres of expertise** to make them more accessible
- **Facilitating digital consultations** which include the patient, the GP, the specialist from a centre of expertise

> Potential Challenges

- **Challenges:** Member states' willingness to share resources, creating a harmonised process, what will incentivise the funding of a state to send a patient abroad, lack of specialists

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CONCLUSION

> Common themes in recommendation:

- Collaboration / harmonisation
- Improved education and training

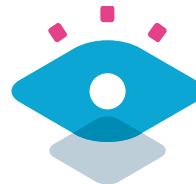
> Group experience/feeling

- Coming from different countries and different backgrounds but we come to similar conclusions
- There is a lot to do before we achieve scenario 3
- The initiative, working with young people, has opened our eyes to the possibility of change we can make

THANK YOU FOR YOUR ATTENTION.



GROUP 2



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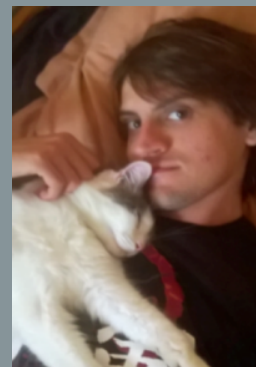
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GROUP 3

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STEFAN ZIVKOVIC
RITA FRANCISCO
THOMAS VILLARET
MARIANA COUTINHO
MARIE BARTH

Young Citizen Conference RECOMMENDATIONS.



INTRODUCTION: IF WE HAD A MAGIC WAND

- We would have thriving partnerships between patients and other stakeholders (clinicians, researchers, policy-makers)
- We would have equal, borderless access to healthcare
- Patient data would be owned by the patient and stored safely (anonym)
- Patient-centred research would be the standard
- Every RD patient would get a quick and accurate diagnosis, and an effective treatment



PART | 2

OUR RECOMMENDATIONS

RECOMMENDATION 1: ENSURE THAT RD PATIENTS HAVE EQUAL ACCESS TO HEALTHCARE

- Why?
 - Inequalities between European countries in access to healthcare for RD patients
 - Differences between private and public sector
 - There is a need to optimize legal framework
- What for?
 - Accelerate communication/cooperation between european countries
 - Avoid delayed diagnosis/access to efficient treatments and care
 - Reduce the financial burden on RD patients and their carers
- How?
 - European guidelines for the management of rare diseases
 - Mixed funds for patients who need specialized treatments abroad
 - Improve the authorisation/payment process to access cross-border healthcare

RECOMMENDATION 2: EDUCATION AND TRAINING OF ALL STAKEHOLDERS TO IMPROVE PARTNERSHIPS

- Why?
 - Foster knowledge and partnerships involving patients
 - Reduce cultural differences and barriers
- What for?
 - Better communication of and focus on patients and stakeholders needs
 - More efficient research
- How (if applicable)?
 - Training for patients, clinicians, researchers and policy-makers
 - Expert patients
 - Common EU medical school program to train doctors (patient rights, cross-border health care)



RECOMMENDATION 3: FACILITATE DATA OWNERSHIP AND PROTECTION

- Why?
 - More digital data is accumulated (big data, EHR)
 - Sensible patient data
 - Access to health care
- What for?
 - Allow more data to be stored safely
 - Accelerate research and make it more efficient
- How (if applicable)?
 - Robust standards for legally and ethically data sharing
 - Patients as legal data owners (decide sharing parties)
 - App for authorisation of personal data use in specific cases

#my_data_my_choice!

PART | 3

CONCLUSION

YOUR PERSONAL CONCLUSION

This experience has been:

"Demanding...Unique... the ultimate ACT now, CHANGE tomorrow exercise" - Rita

"This is a prime example of what it means to 'be ahead of the game' and how we are more prepared for what is coming if we stand together and have an active role in the decisions that concern us. I am truly grateful for everything I learned with this enriching experience"
- Mariana



Behind every crazy dream,
There is a worthy decision.



"Innovative Idea-Factory to improve the future of rare disease patients" - Marie

THANK YOU FOR YOUR ATTENTION.



GROUP 3



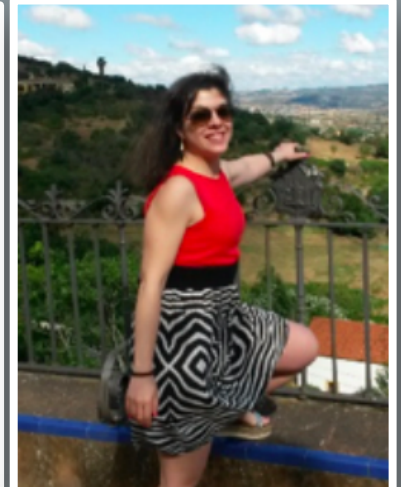
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Foresight in Rare Disease Policy



CATERINA DALLAI, Psychology Student, Italy
ORIANA DE SOUSA, Patient & Psychologist, Portugal
HAYLEY HUBBERSTEY, Real World Evidence Manager &
Patient advocacy group chair, UK
ANETA MORKOVSKA, Patient & Doctor, Czechia

Young Citizen Conference RECOMMENDATIONS



INTRODUCTION:

> DIAGNOSTICS:

- > Shift from relative ignorance of the issue towards general awareness of the diagnostic issue in RDs (awareness amongst doctors)

> INTERGRATED, SOCIAL, HOLISTIC CARE:

- > Recognising the role of health care providers and other social services (particularly those involved in e.g. European Reference Networks) in achieving these goals



PART | 2

OUR RECOMMENDATIONS

RECOMMENDATION 1: DIAGNOSTICS

> Why? Issue & groups involved

- **ISSUE:** Cost of developing test, Doctors skills to dedicate, not enough incentives, rolling out tests and communication
- **GROUPS INVOLVED:** Healthcare system from local hospitals to international networks

> What/how?

- **RECOMMENDATION:** Training and education of networks of doctors on the availability of test and devices provided in conjunction with patient associations
 - Patient associations to promote partnerships with academic institutions programme. Working with academic institutions for training for health care professionals making sure rare disease is on the teaching agenda early on
 - Organisation of annual virtual meetings for key groups and doctors, increased coordination between ERNs and hospitals to develop protocols and flowchart of symptoms to consider rare diseases
- **LEVEL OF ACTION:** National and European
- **CHALLENGES:** Low cost but high efforts in coordination and continuing to get the message out

RECOMMENDATION 2: INTERGRATED, SOCIAL, HOLISTIC CARE

> Why?

- **ISSUE:** Doctors don't often assess non clinical needs/daily needs as they are already very busy with their schedule and unaware of the intricacies of rare disease
- **GROUPS INVOLVED:** Case managers, Social workers, Doctors network, Psychologists, Patient organisations, Patient advocates & experts, other HCPs, Academics & Health economists.

> What/how?

- **RECOMMENDATION:** Bring social care into the healthcare system by creating coordination type roles to bridge the gap within countries and across the EU. This is followed up with research on holistic care to generate evidence of importance for ongoing support.
 - By sharing studies which already exist and have worked well on a national level and lobby to move to EU level to gain funding.
 - Expand the scope of ERNs to include both integrated and social holistic care and include a larger group of stakeholders all together
 - Continue to talk and include patients and caregivers to fully understand the needs
 - Raise awareness of the resources already available and work with rare disease organisations
- **LEVEL OF ACTION:** National and European
- **CHALLENGES:** High cost, might be difficult to convince governments but the data generated in the long term will support its worth

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CONCLUSION

> Common themes in recommendation

- Multi-disciplinary teams
 - Keeping the patient voice, patient experts and patient associations centre front
 - Better use of ERNs or creating central clinics/units for rare disease in Europe (one stop shop) working with or in hospitals directly
 - Providing data or education to continue learnings more about rare disease and the benefits of this work
 - Cost is always a barrier and find solutions around this, the cheaper less perfect options, or joint solutions where countries can work together to share the costs.
 - A need for providing evidence on the benefits vs cost of a new approach vs long term cost of not implementing anything, a need to work with academics and health economists on generating data.
 - Doctors and HCPs time is always a barrier so lets make things as straight forward as possible and keep sending the message out
- > As a group we really enjoyed working together and we all had different experiences. We had patients, students, doctors and researchers in our group all from different countries in Europe. This enabled us to form well considered and well formed ideas.
- > We identified a key theme of the doctor-patient model and a paradigm shift in communication because a patients quality of life is more than just the clinical aspects of the condition

THANK YOU FOR YOUR ATTENTION.

- CATERINA DALLAI, Psychology Student, Italy
- ORIANA DE SOUSA, Patient & Psychologist, Portugal
- HAYLEY HUBBERSTEY, Real World Evidence Manager & Patient advocacy group chair, UK
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