Drug Approval and Market Access
Can Patients make a difference?

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The Role of the Regulators in Drugs for Rare Diseases

European Medicines Agency – guarantors of probity

- Quality
- Safety
- Efficacy

Traditional model – pre-clinical development (proof of concept, toxicity etc) before three phase clinical trial process

- Phase 1 – safety
- Phase 2 – dose
- Phase 3 - efficacy

The Role of the Regulator #2

Traditional model doesn't work for rare diseases – too few patients, too widely dispersed, costs too high for likely return.

But patients and families need to know that new drugs are safe and effective.

Stimulus needed to encourage drug development in Europe

- Orphan Medicinal Products Regulations
- Advanced Therapy Medicinal Products Regulations

Patient and Family Input to EMA decisions

Scientific Committee Membership

- COMP – determines if the disease is rare and if the proposed early idea is plausible
- CAT – for gene, stem cell and tissue engineered products
- PDCO – medicines for children
- Scientific Advice and Protocol Assistance
- CHMP – Market Authorisation

Patient and family input to all stages as members and/or advisors

- Define the condition
- What matters, not just what can be counted
- Is the development plan acceptable
- Is the benefit/risk calculation appropriate

EMA plan for 2020-2025 – much greater input from patients and families

From Market Authorisation to Patient Access

- MA – “you can sell it”
- HTA (Health Technology Assessment) – “does it make a big enough difference?”
- Pricing and Reimbursement – “do we want to buy it?”

The best drug in the world is useless if it never leaves the pharmacy shelf!

How can patients and families influence access?

MA is EU wide, HTA and P&R are national so systems and practices vary (but growing tendency to harmonisation)

HTA and P&R making resource allocation decisions in a constrained environment

Patients and families have the lived experience of the condition

- Rare diseases often poorly characterised
- Mental and physical
- Multi system
- Family impact
- Genetic
- Turn numbers into impact

The Role of Patient Organisations

Build critical mass – from anecdote to evidence

Support and training for members so they can participate in the process effectively

Advocacy and lobbying for access and improved support for those affected

Awareness raising for public and professionals

Work with industry to improve trial design and boost recruitment and retention

Be Positive

Research progress – rare diseases have greater priority thanks to patients and families advocacy

New scientific and clinical opportunities

genomics, bioinformatics, genome editing, stem cells etc.

Growing power of the patient voice in decision making

And finally, always remember that we are not alone……..

- “Coming together is a beginning. Keeping together is progress. Working together is success” *Henry Ford*
- Nihil de nobis sine nobis (Polish constitution -1505)
- “Alone we can do so little, together we can do so much” *Helen Keller*
- “Because we are (all) worth it” *after L’Oreal*
- “Yes we can” *Barack Obama*

Thank you

for listening

Any questions?