VSOP

Alliance for Rare and Genetic disorders – the Netherlands

Patients´ association perspective

Guideline for registry-based studies

EMA 19th October 2020

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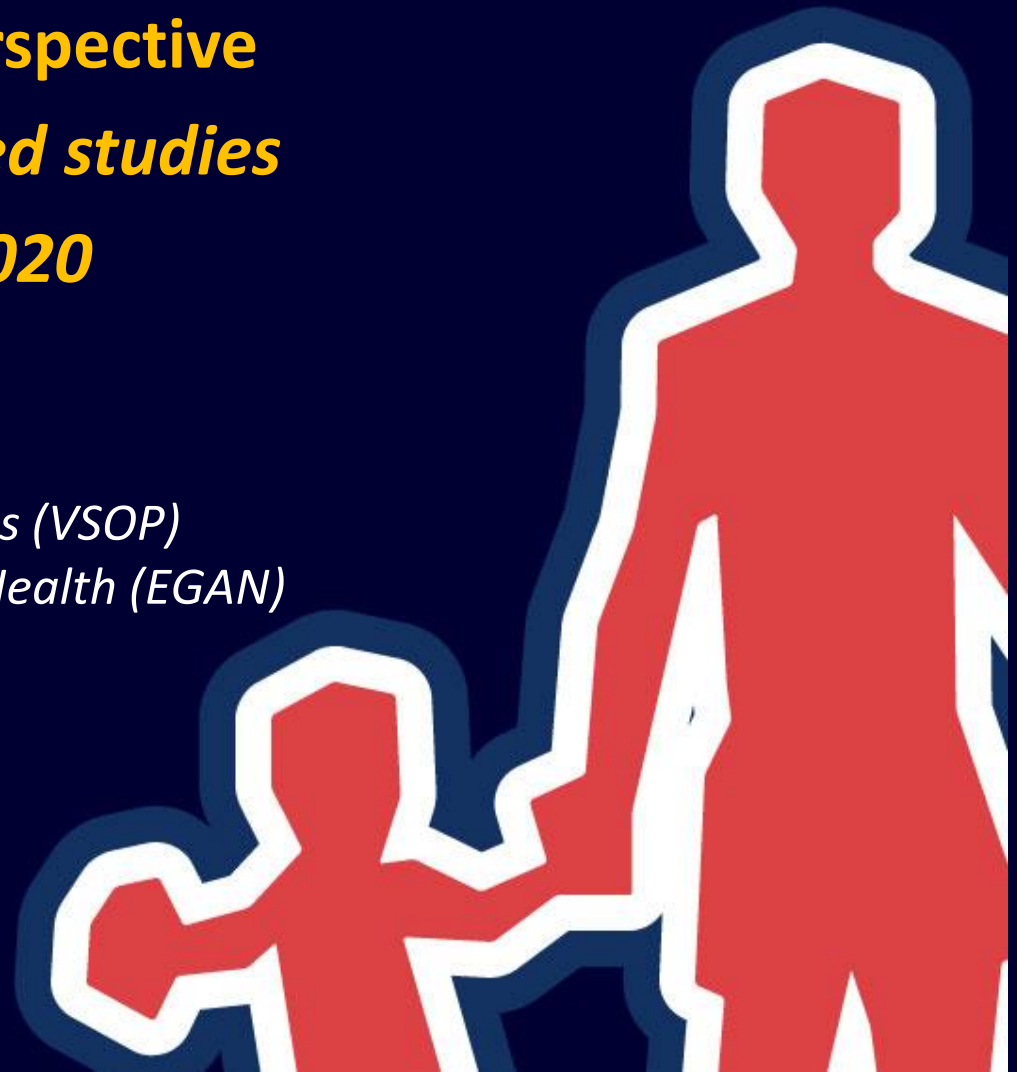

VSOP

Patient Registry & Registry Studies

- Early data collection, Natural history (PRIME)
- Patients eligible for clinical trials
- Monitoring outcomes of treatment (PAES, PASS)

e.g. Long Term Follow Up Gene Therapy

- Normal pharmacovigilance vs PASS
- Monitoring quality of care
- Information for policy making

VSOP

Patient as participant

- Informed (dynamic) consent & validity for studies
- Privacy – GDPR – anonymisation, pseudonimisation-unique identifier EUPID
- identifiable data (small numbers ultra rare diseases)
- Where are the data stored?
- Results from studies reported back to participants
- Transparancy, support base, trust

Patient as data source

What is in it for me?

- Patient reported outcomes, quality of life (questionnaires), remote trials, tele-monitoring
- Adverse events, side effects, reason of stopping
- Adherence
- Access to expensive medicines (HTA)
- What is the best medicine for me?
- Link to biobank

Improve insight disease.

How am I doing, compared to others?

The Increasing Focus on the Patient in Patient Registries

Registries for Evaluating Patient Outcomes: A User's Guide: 4th Edition

Patient organisation

Why is engagement important?

- Information, education & support base
- Formal role in governance, management of registry e.g. board and steering group
- Finance, acquisition of funding
- Co-creation & Partnership
- Rights & Obligations

Patientenregisters.org

Developments

EMA Qualification – very few qualified

Cystic Fibrosis, CAR T, Multiple Sclerosis

- *Feasibility analysis by MAH*
- *multiple products, multiple MAHs*

EU Platform Rare Disease Registration

24 European Reference Networks

Data strategy - Eurordis

***Hemophilia** - World Federation of*

WFH Gene Therapy Registry

Developments NL

Zorginstituut Nederland

Managing patient registries for expensive drugs

(cost)-effectiveness and (adverse) effects in practice

4 pilot projects 2021-2

2023

Structural funding

Qualification of registries

Evaluation reimbursed medicines

Zorginstituut Nederland

Nieuwsbrief

Regie op Registers

Zorginstituut Nederland | september 2020, nr. 3

Voorwoord

Op 21 september wordt bekend welke casestudie het spits afbijt in het traject Regie op Registers. Een mijlpaal, aldus projectleider Anke ter Horst en Zorginstituut-bestuurder Peter Siebers. "Nu kunnen we zien of wat we samen met de veldpartijen hebben bedacht, ook werkt", zegt Siebers. "Ik ben heel nieuwsgierig naar de ervaringen bij de casestudie."

In de casestudies worden de producten getest die het projectteam het afgelopen jaar in drie deelprojecten heeft ontwikkeld. "De eerste fase hebben we mede dankzij de inzet van de betrokken partijen goed kunnen afronden", vertelt Ter Horst. "In juni hebben we van het ministerie van VWS

leggen om systematisch te kijken wat een medicijn doet in de praktijk. We laten sommige geneesmiddelen voorwaardelijk toe tot het vergoede pakket. Nu kunnen we in de praktijk bewijs verzamelen over de werking."

Goede discussies

Anke ter Horst

Guideline for registry-based studies

Horizonscanning & Priority setting