“Rapid” COS Development for Emerging Therapies

COMET VII, Amsterdam, Netherlands
Green Park Collaborative

- Multi-stakeholder forum to develop guidance for clinical studies intended to inform clinical and market access decisions
  - All projects jointly led with patient groups
  - Active involvement of public and private payers and HTA groups
  - Includes clinicians, regulators, industry, researchers, etc.
coreHEM: Objectives

- Establish a “core set” of outcomes to measure the effectiveness and value of gene therapy in hemophilia
- Seek consensus across all stakeholders
- Process gives preference to patient input
- Project timeline driven by upcoming pivotal trials
coreHEM Sponsors / Partners

National Hemophilia Foundation
for all bleeding disorders

Bayer
Biomarin
Pfizer
Shire
Spark Therapeutics
St. Jude Children's Research Hospital
uniQure

OBSERVERS

Alnylam
novo Nordisk
Roche
Genentech

coreHEM
coreHEM Stakeholders

- clinicians
- government reps
- industry sponsor reps
- US payers
- international payers/HTA
- patients/patient advocates
- methods and epidemiology experts
- academic gene therapy research reps
EXAMPLE OF OUTCOME RETAINED DUE TO PATIENT IMPORTANCE
### Core Outcome Set
- Frequency of bleeds
- Factor activity level
- Duration of expression
- Chronic pain
- Mental health status
- Utilization of healthcare system (direct costs)

### Additional Outcomes
- Duration/frequency/type of physical activity/sport/play
- Physical health/general health perception

### Adverse Events

#### Short-Term
- Liver toxicity
- Short-term immune response to FVIII/FIX
- Immune response to gene therapy (cytotoxic)
- Thrombosis

#### Long-Term
- Development of other disorders
- Vector integration into host genome
- Duration of vector-neutralizing response

#### Mortality
- Cause of death
Core outcome set for gene therapy in haemophilia: Results of the coreHEM multistakeholder project

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Payer / HTA Recognition

• Statement below appeared in final report:

  o “We will consider this recommended core outcomes set...in future assessments of gene therapies for hemophilia”
  o We would encourage….researchers to consider this recommended core outcomes set when developing study protocols...