‘Patient perspective’

Session 3: Fundamental challenges in small populations

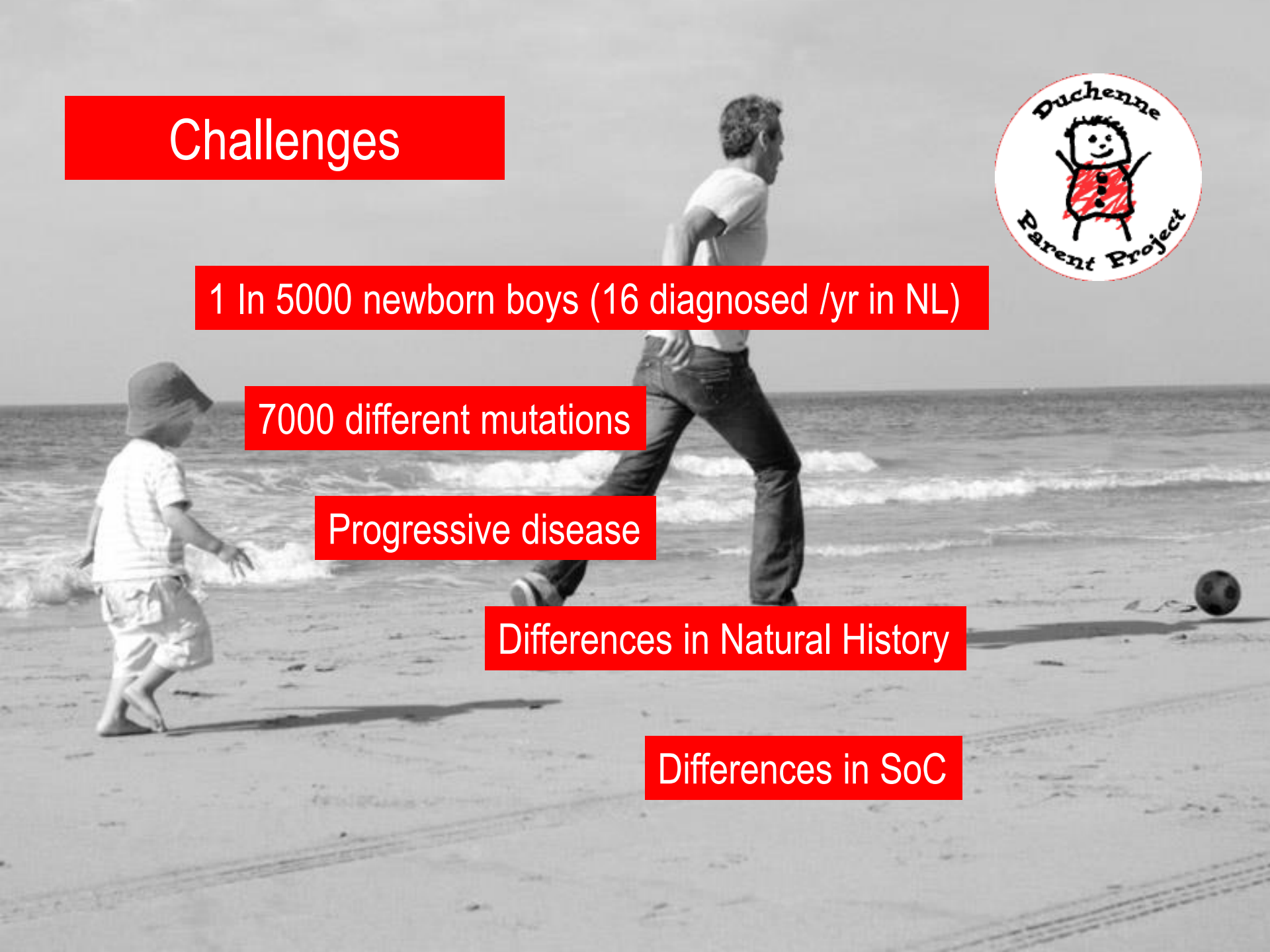
Correct evidence: The role of randomization and observational data

Elizabeth Vroom

Asterix Conference I Zaandam I 18-19 September 2017

Hopes, concerns and requirements

'Towards optimal trial design which can lead to reliable outcomes and conclusions'


Challenges
1 In 5000 newborn boys (16 diagnosed /yr in NL)
7000 different mutations
Progressive disease
Differences in Natural History
Differences in SoC
Duchenne
Parent Project

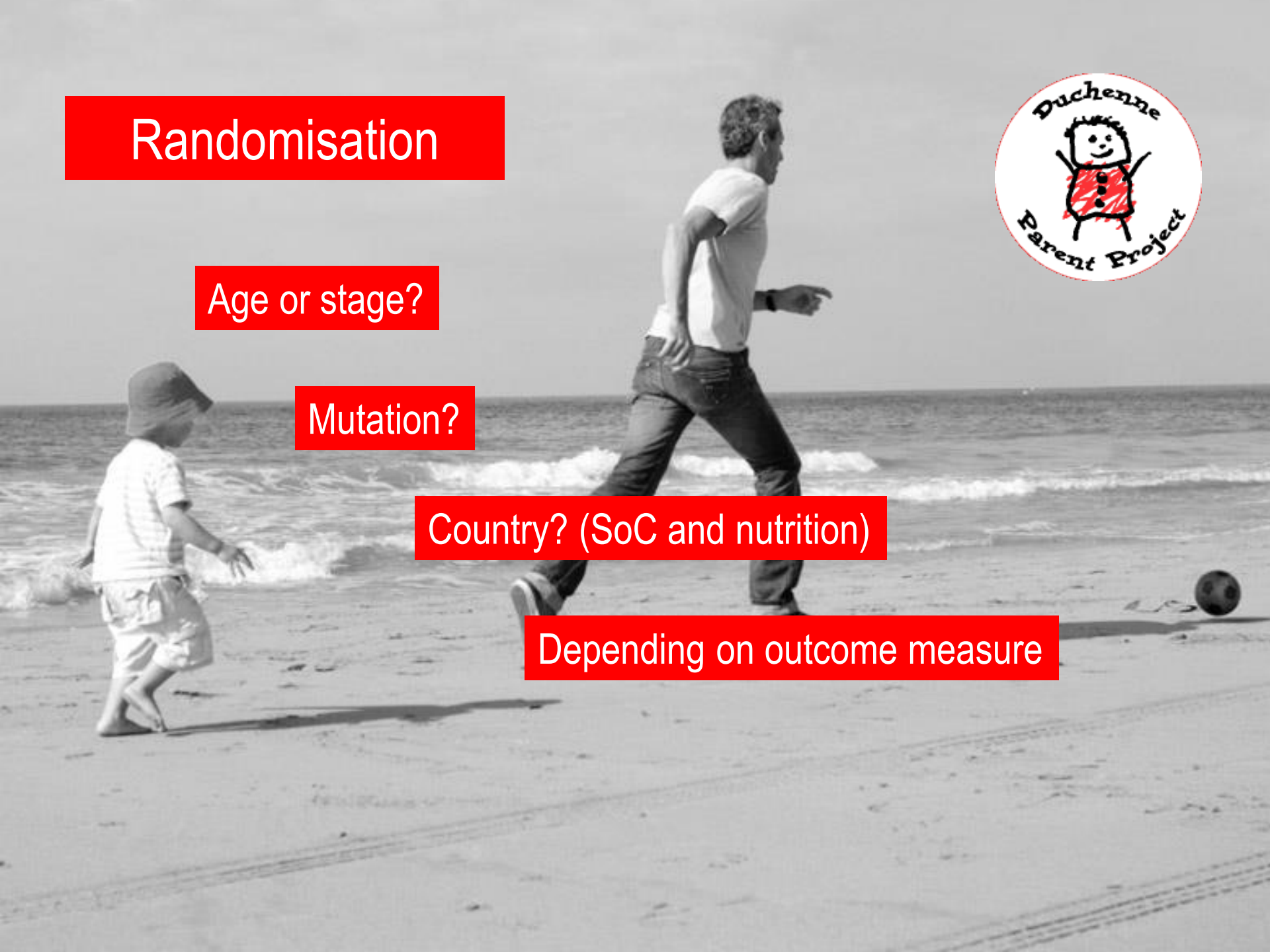
Randomisation

Age or stage?

Mutation?

Country? (SoC and nutrition)

Depending on outcome measure




Observational
Evidence is considered ‘low’
Market Authorisation and HTA
Use of natural history controls is limited
Duchenne
Parent Project

Natural History controls (registries)

Useful when drug effect is ‘big’

Lack of placebo effect

Sharing data of placebo groups from other trials

Natural history data are fast ‘outdated’


Thank you!