DUCHENNE MUSCULAR DYSTROPHY
WHAT A COMMUNITY CAN DO

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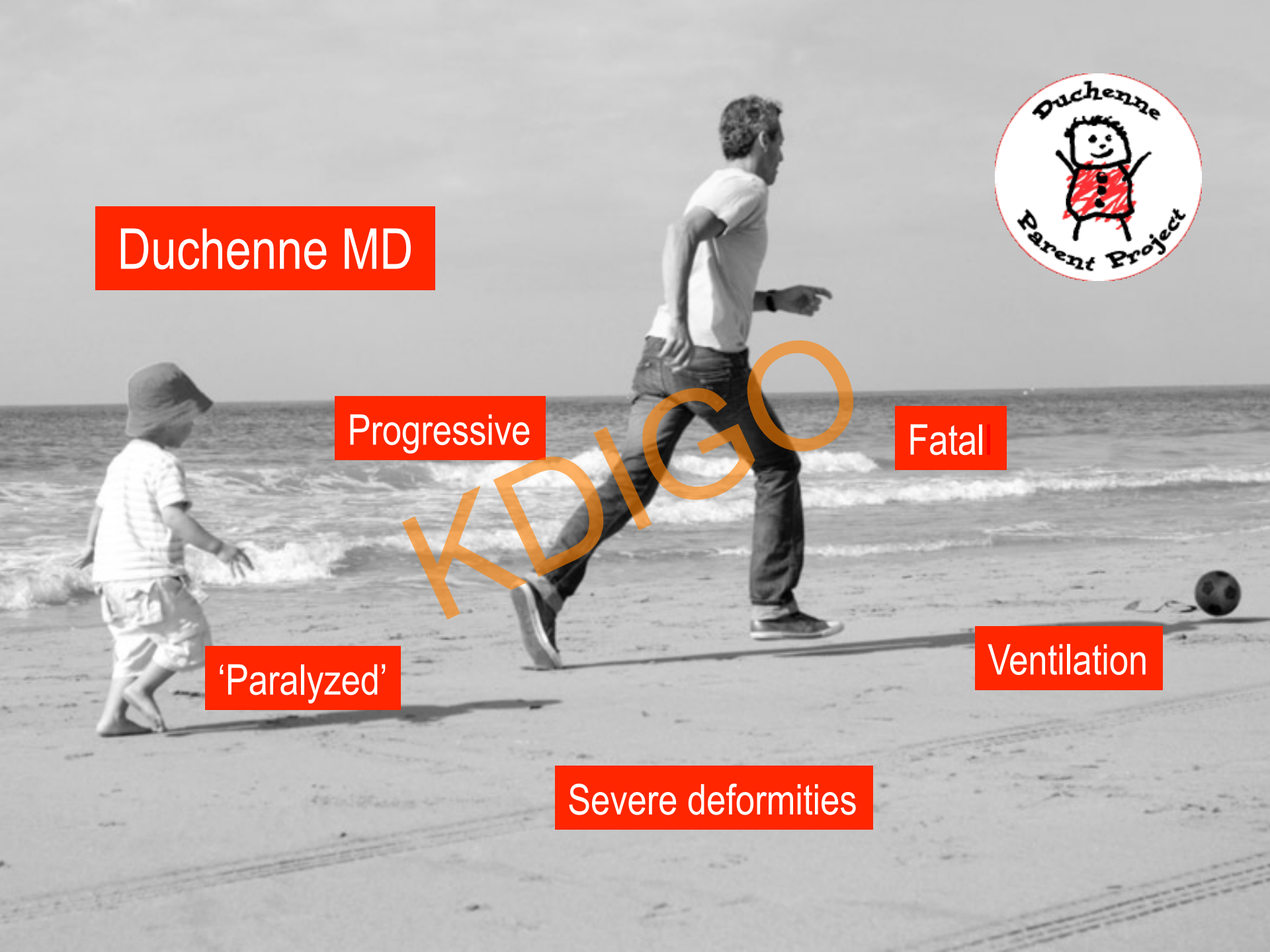
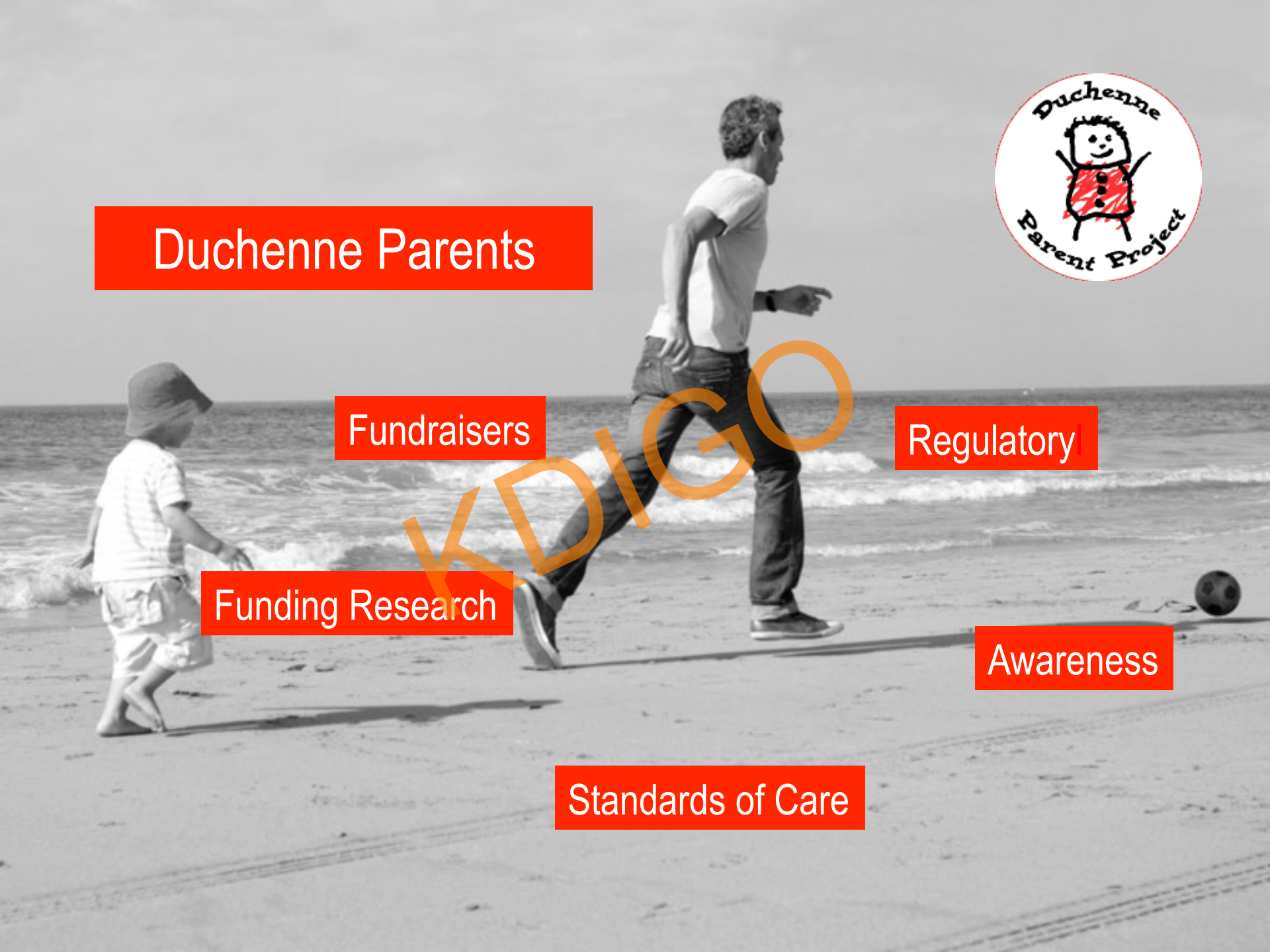
Disclosure of Interests

President Duchenne Parent Project Netherlands

Chair United Parent Projects MD


Duchenne MD
Progressive
Fatal
KDIGO
‘Paralyzed’
Ventilation
Severe deformities
Duchenne
Parent Project


Duchenne Parents
Fundraisers
Regulatory
KDIGO
Funding Research
Awareness
Standards of Care
Duchenne
Parent Project


Regulatory
Guidelines
KDIGO
Benefit/Risk
Workshops
Outcome measures
Policy paper
Duchenne
Parent Project


KDIGO

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Stakeholder cooperation to overcome challenges in orphan medicine development: the example of Duchenne muscular dystrophy

Volker Straub, Pavel Balabanov, Kate Bushby, Monica Ensini, Nathalie Goemans, Annamaria De Luca, Alejandra Pereda, Robert Hemmings, Giles Campion, Edward Kaye, Virginia Arechavala-Gomeza, Aurelie Goyenvalle, Erik Niks, Olav Veldhuizen, Pat Furlong, Violeta Stoyanova-Beninska, Matthew J Wood, Alex Johnson, Eugenio Mercuri, Francesco Muntoni, Bruno Sepodes, Manuel Haas, Elizabeth Vroom, Annemieke Aartsma-Rus

Take home messages

A collaborative and constructive dialogue between patients' representatives, academics, industry, and regulators can facilitate and accelerate treatment development for rare diseases

Take home messages

For rare diseases, development and implementation of standards of care to decrease variability is crucial for multicentre trials

Take home messages

Functional and molecular outcome measures should be developed in collaboration with patients' representatives and regulators

Take home messages

High-quality data for natural history and outcome measures are crucial for clinical trial design and regulatory approval; ideally, data should be obtained before or in parallel with potential treatments

Take home messages

Developing a treatment for a rare disease such as Duchenne muscular dystrophy should be a global effort. Regulatory requirements should be aligned and communication should be continuous between regulatory bodies in different global regions with respect to guidelines for treatment development and biomarker qualification.