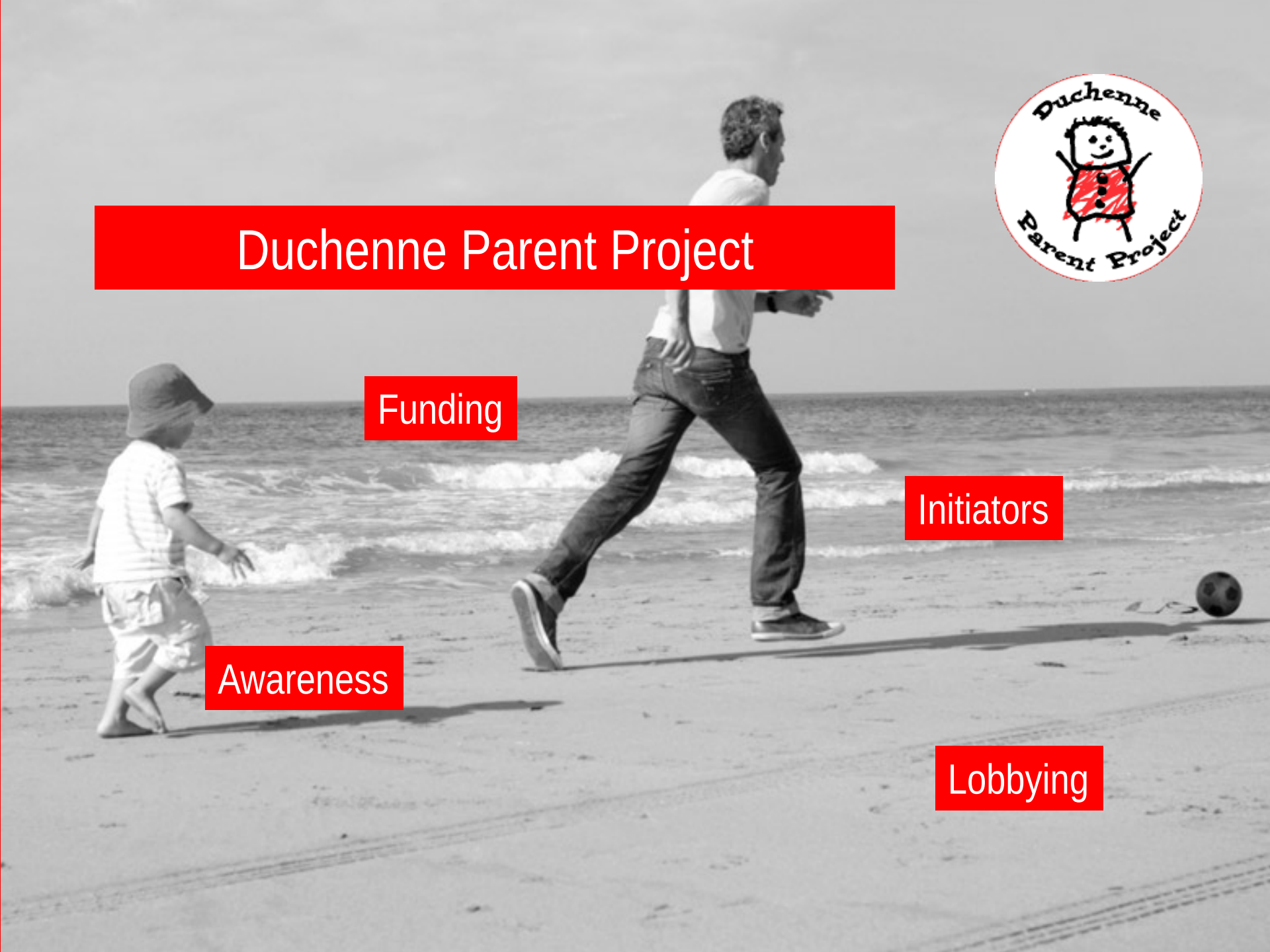
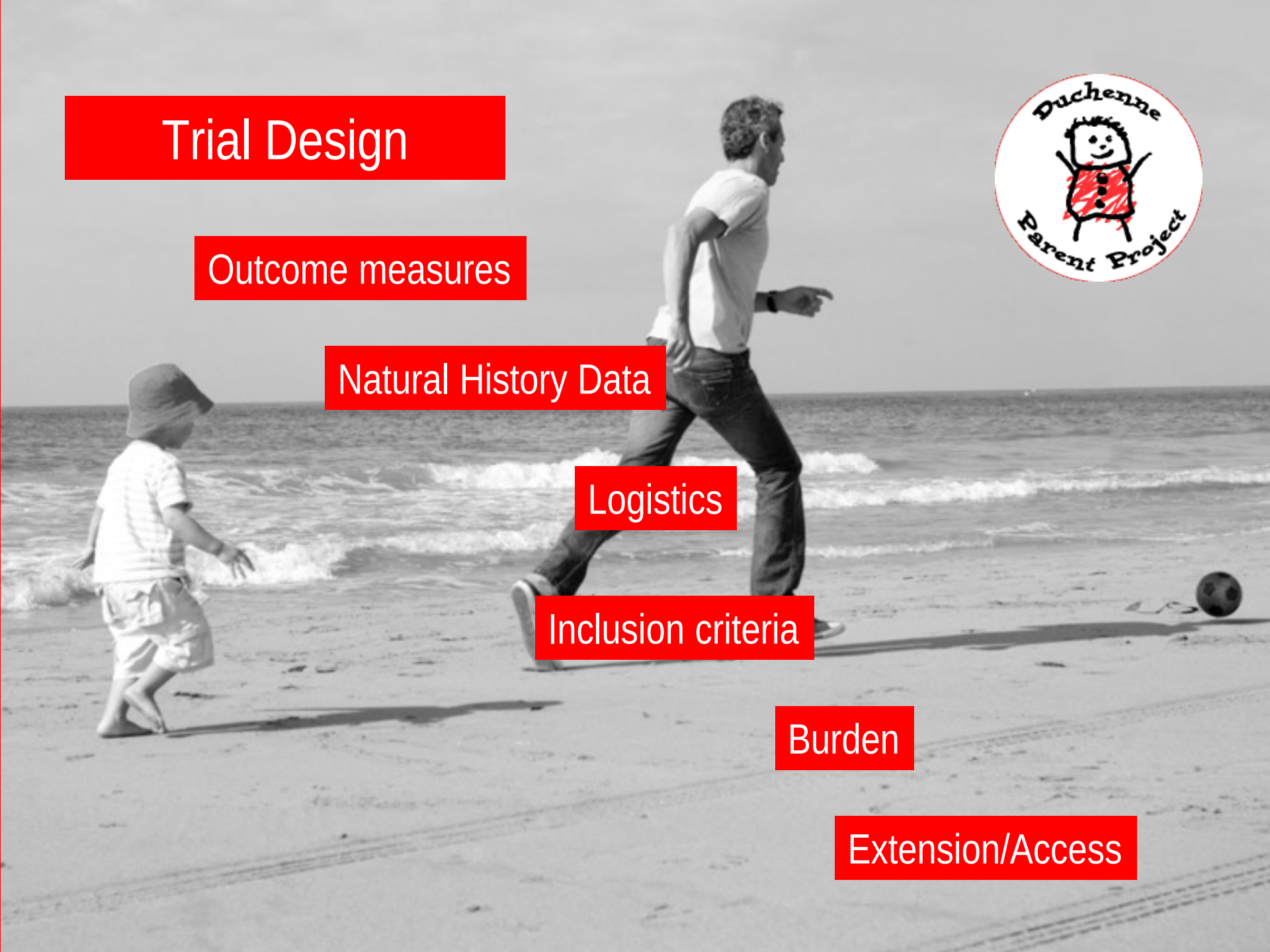
Role of patient organisations in research

Elizabeth Vroom, Budapest 18/4/2013


Patient organisations
Universities
Industry


Duchenne Parent Project
Funding
Initiators
Awareness
Lobbying
Duchenne
Parent Project


Clinical research
Trial Design
Selection of Centers
Recruitement
Collaboration with Industry
Regulatory
Ethics
Duchenne
Parent Project


Trial Design
Outcome measures
Natural History Data
Logistics
Inclusion criteria
Burden
Extension/Access
Duchenne
Parent Project

Outcome measures

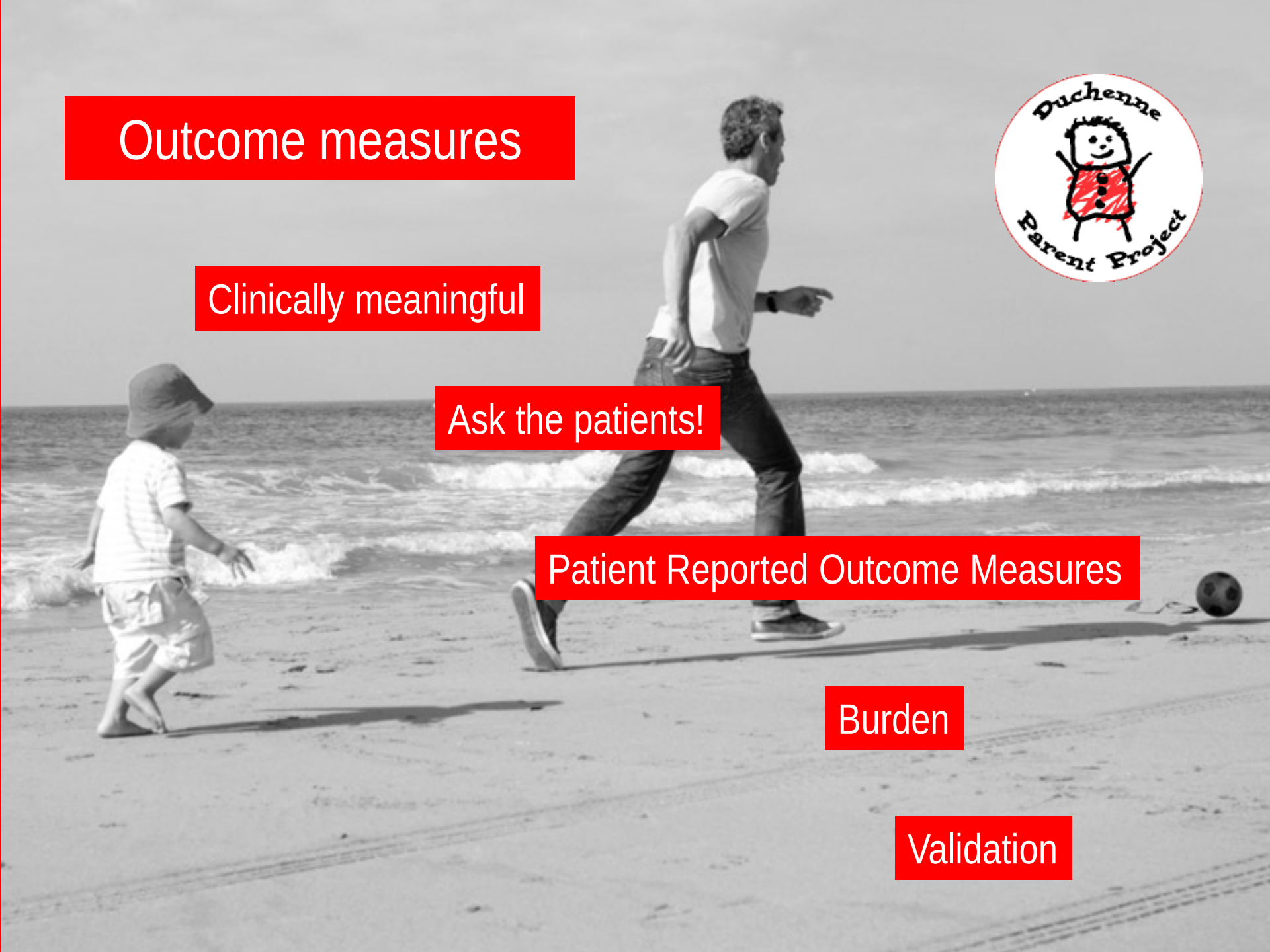
Clinically meaningful

Ask the patients!

Patient Reported Outcome Measures

Burden

Validation

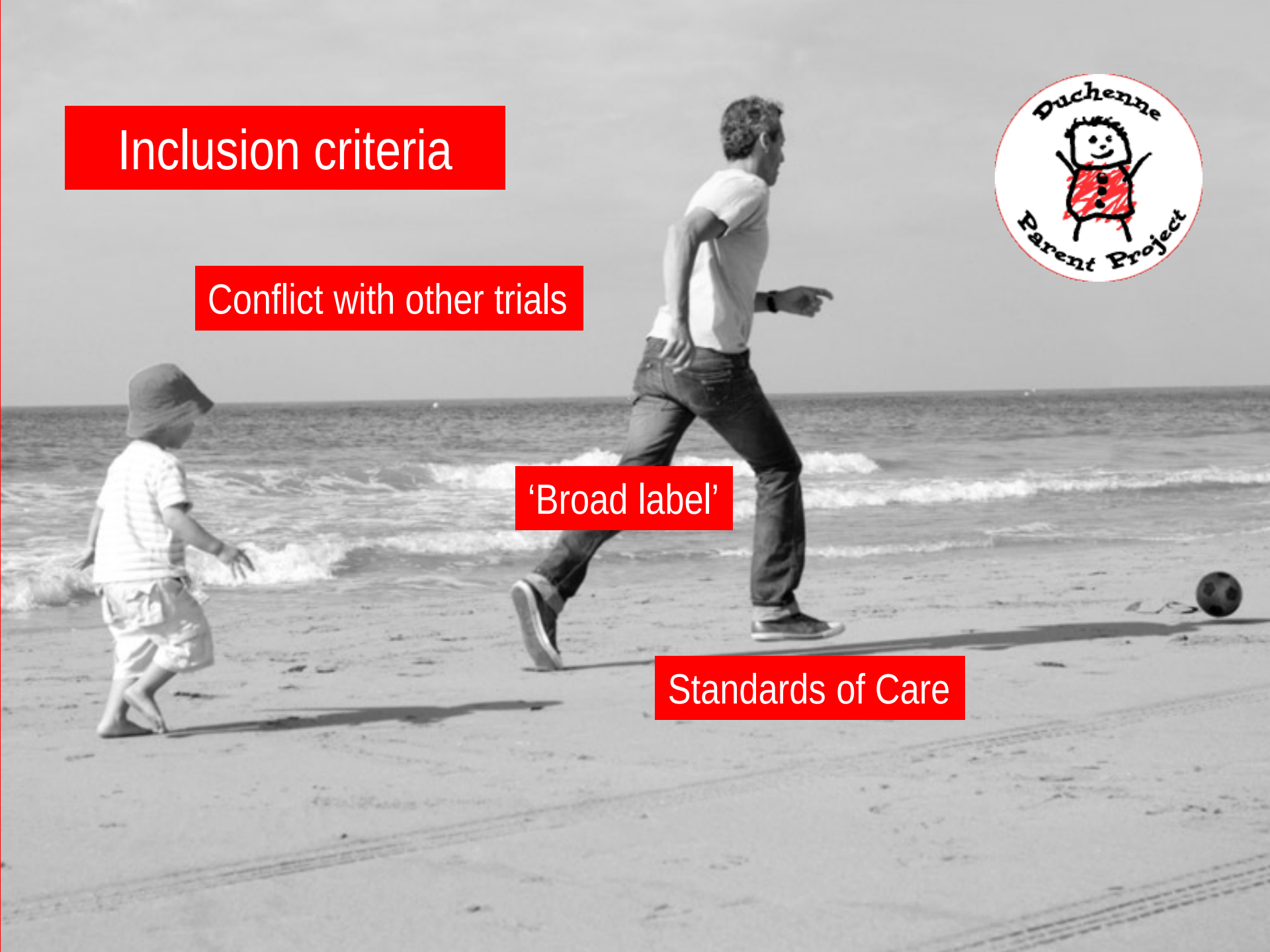


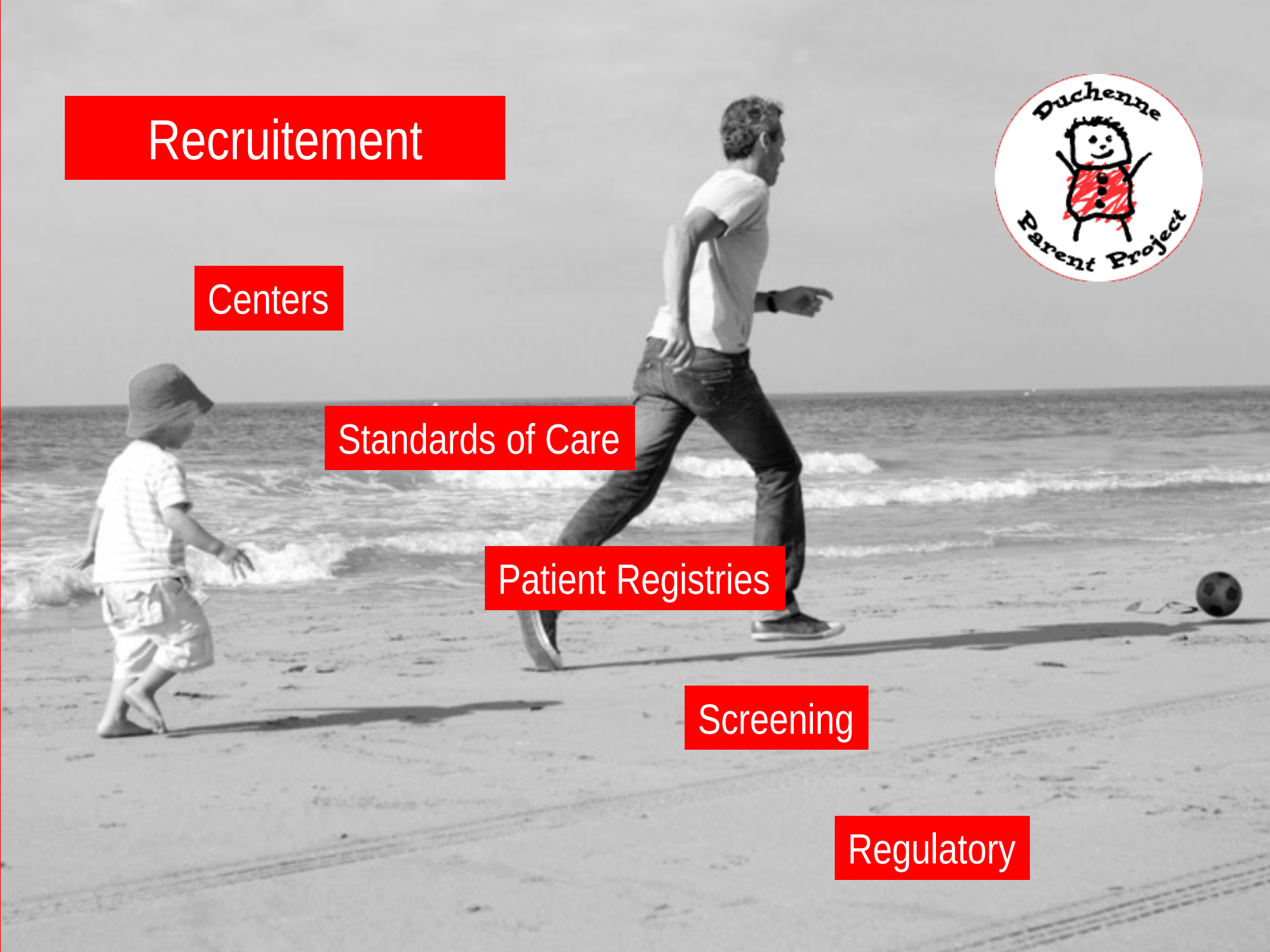
Inclusion criteria

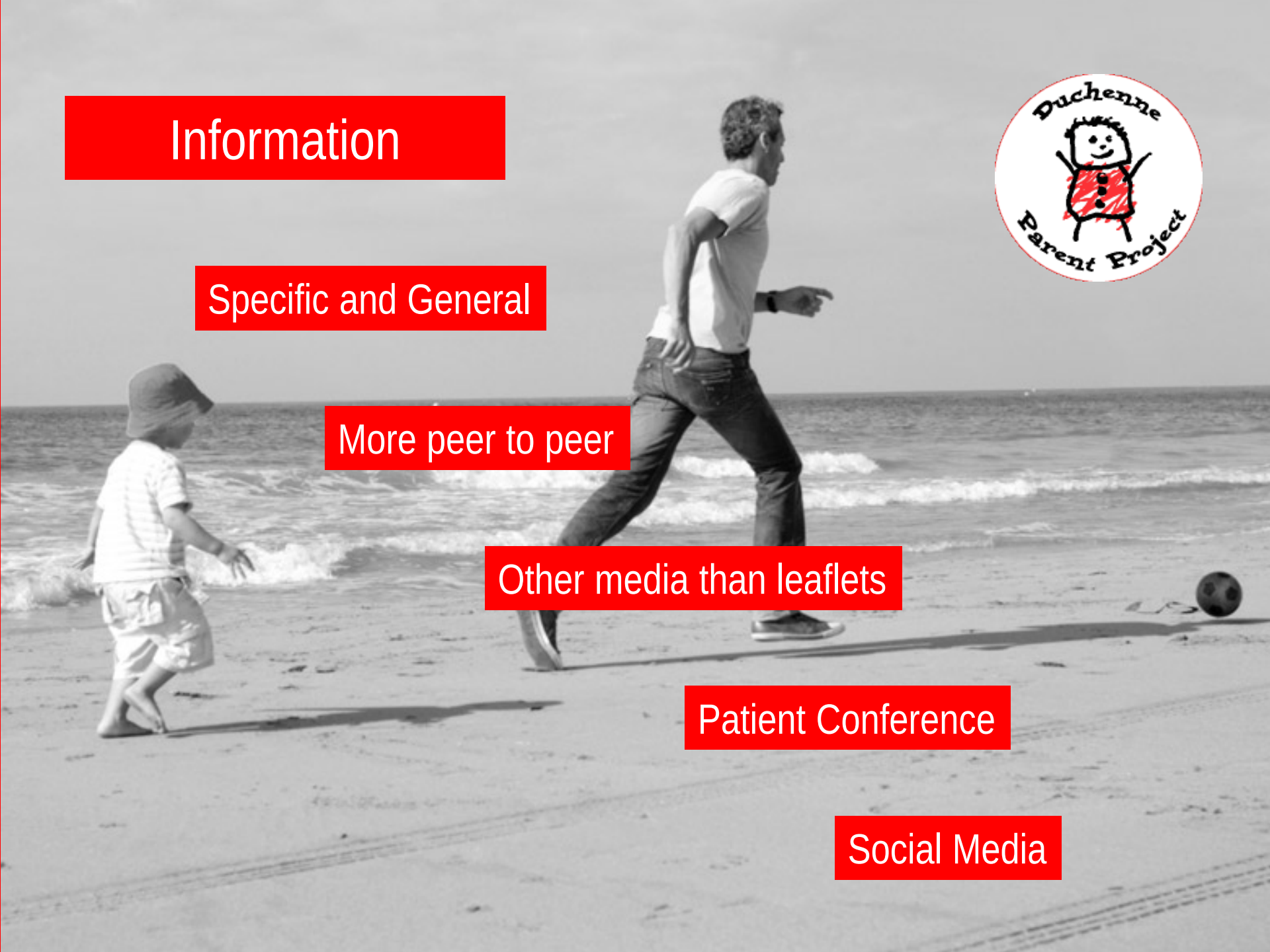
Conflict with other trials

‘Broad label’

Standards of Care




Recruitement
Centers
Standards of Care
Patient Registries
Screening
Regulatory
Duchenne
Parent Project


Information
Specific and General
More peer to peer
Other media than leaflets
Patient Conference
Social Media
Duchenne
Parent Project

Home, Children and Clinical Studies, NHLBI, NIH, DHHS

http://www.nhlbi.nih.gov/childrenandclinicalstudies/index.php

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Children AND CLINICAL STUDIES

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No More Hand-Me-Down Research

Importance of Research In Kids

Why is research important, how is it different, safety and protections, are there benefits, your right to say no

Find out more

Getting Started In a Study

Information on the research team, what you might need to ask, and what role kids play in participating

Find out more

Download Video Options

Once In a Study

Information on how studies affect the family and what kids think, what happens if you leave a study or what happens when it ends

Find out more

Resources

Know what rights you have, where to find information, and terms that you may hear in a study

Find out more

Show Video Transcript

Children have often had to accept medicines and treatments based on what is known to work in adults. As a society, we should not agree to this "hand-me-down" approach.

General Principles (1)

Patient organisations (POs) should be informed of all aspects of the clinical protocol before collaboration.

POs should actively contribute to the documents aimed at patients - patient information document and the informed consent form.

Areas of and extent of collaboration should be enumerated in the “Agreement of Understanding”, available to all stakeholders: patients, sponsors, investigators, ethics committees and national competent authorities.

Financial relationships (ie, between sponsors and Pos) are transparent.

EURORDIS
European Organisation for Rare Diseases

General Principles (2)

Study results should be published, even in case of negative outcomes, non-conclusive or otherwise abandoned clinical trials.

Data acquired during clinical trials should be made available to the scientific community, with a view to fostering scientific progress and avoiding unethical duplication of clinical trials.

The commitment of a PO in the design and/or development of a trial does not modify the role and responsibilities of the sponsor, even if the study is financially supported by the PO.

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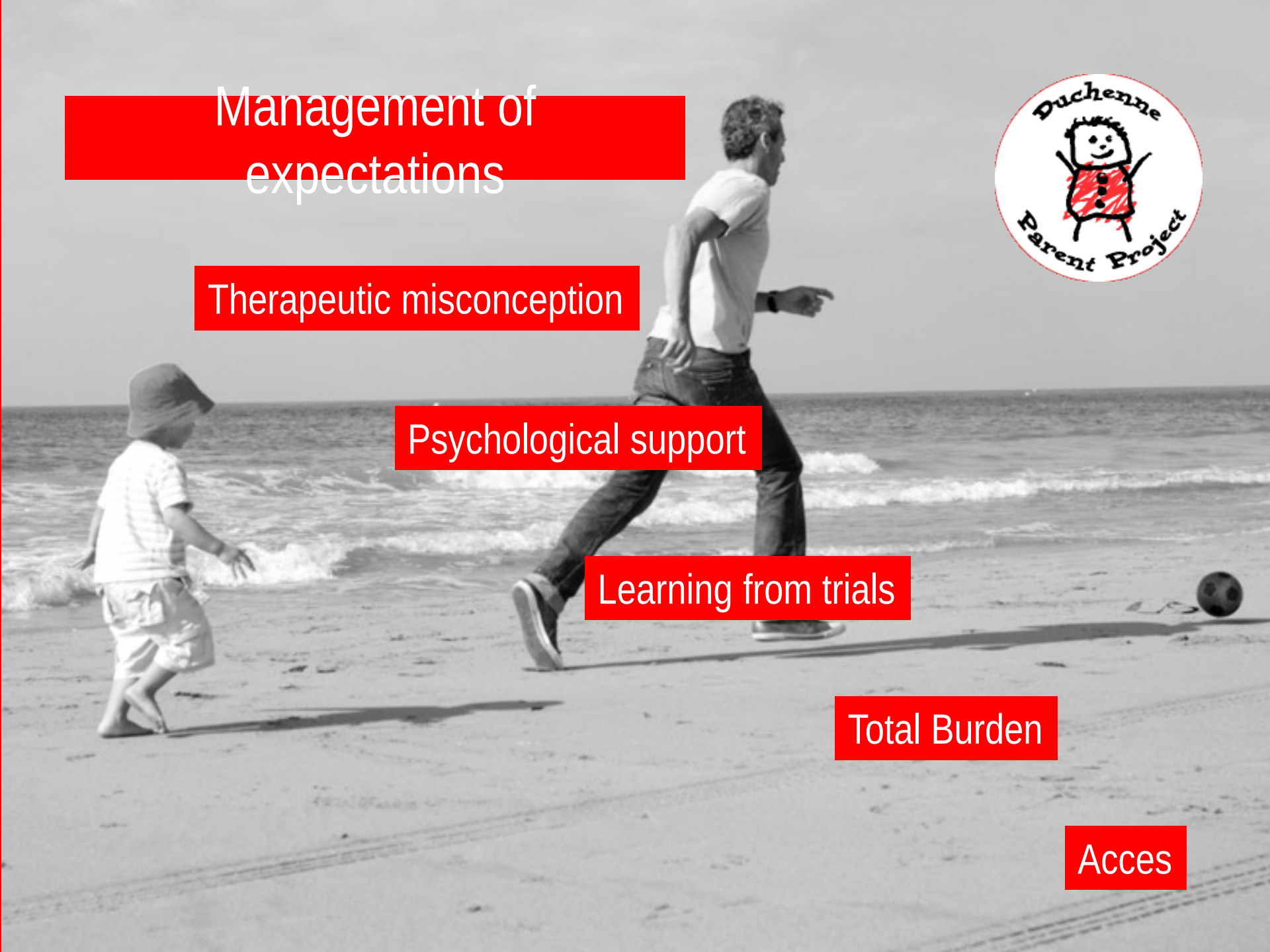
European Organisation for Rare Diseases


Management of expectations
Therapeutic misconception
Psychological support
Learning from trials
Total Burden
Acces
Duchenne Parent Project


Include patients and PO’s at an early stage!


Thank you!