Timely completion of Paediatric Investigation Plans

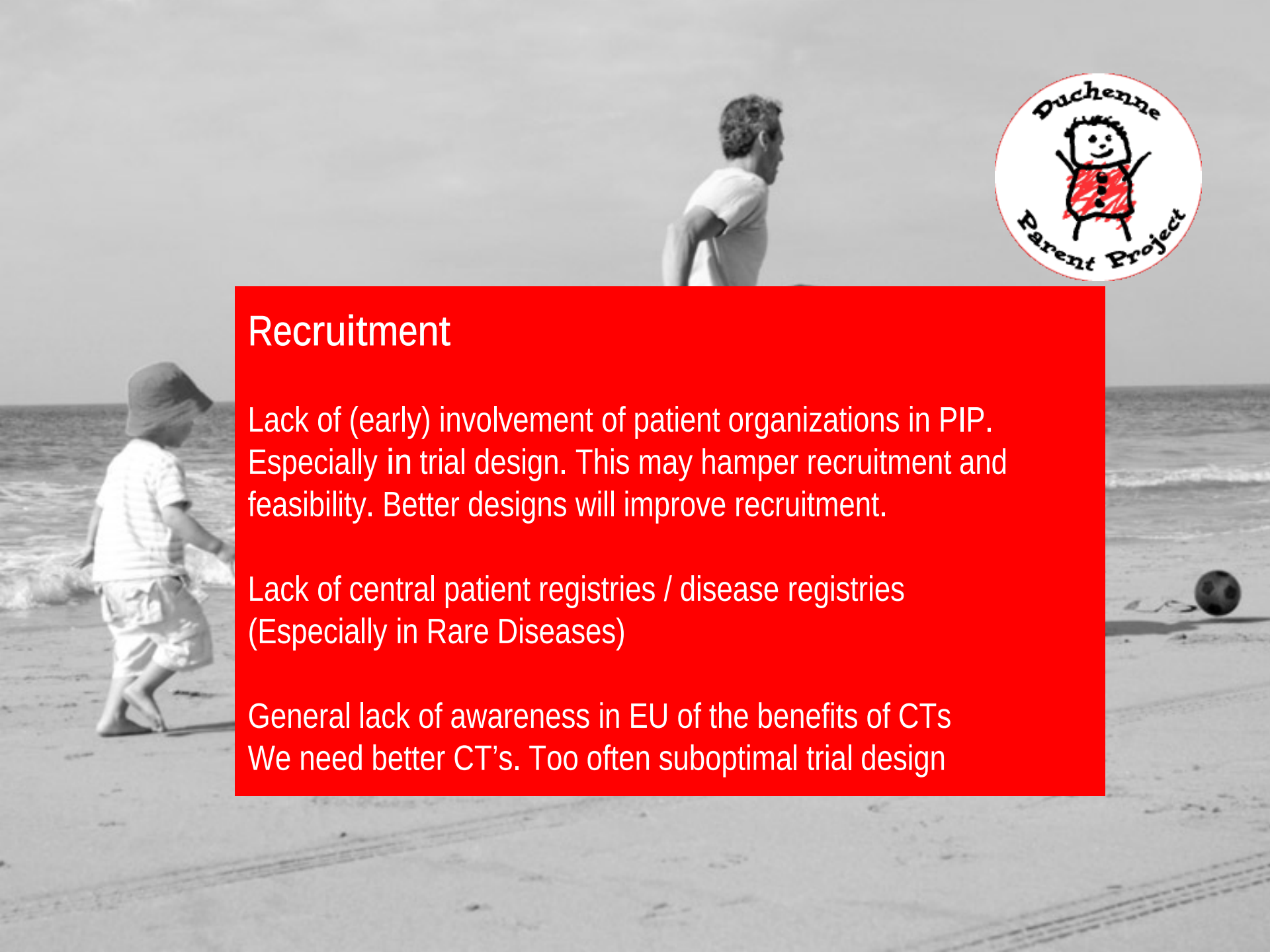
EMA/EC multi-stakeholder workshop March 20 2018

Elizabeth Vroom, World Duchenne Organization

Approval

Different interpretation of Pediatric Regulation by National Ethic Bodies.

Need for consensus and guidelines.

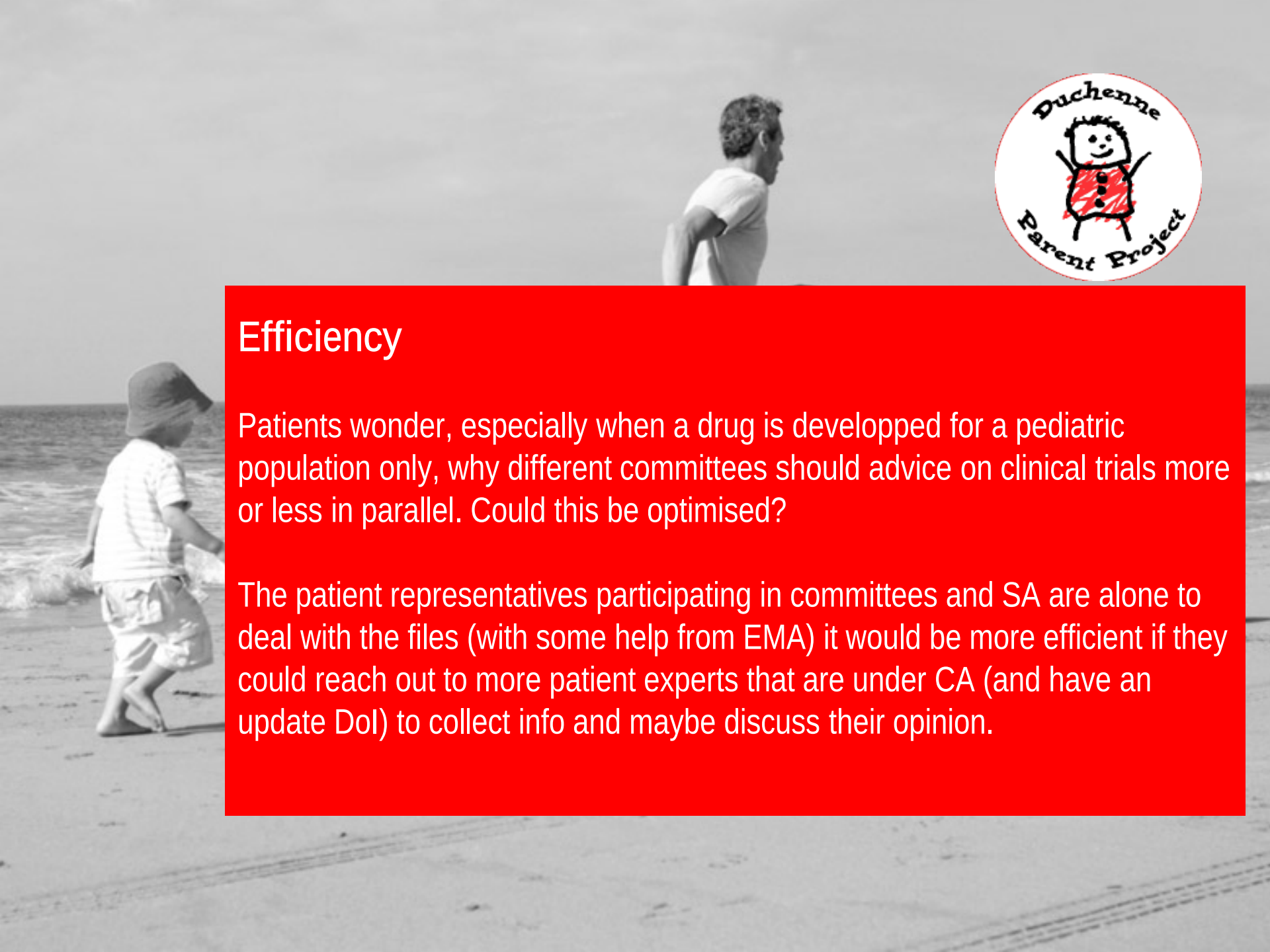


Recruitment

Lack of (early) involvement of patient organizations in PIP. Especially in trial design. This may hamper recruitment and feasibility. Better designs will improve recruitment.

Lack of central patient registries / disease registries (Especially in Rare Diseases)

General lack of awareness in EU of the benefits of CTs
We need better CT's. Too often suboptimal trial design



Efficiency

Patients wonder, especially when a drug is developped for a pediatric population only, why different committees should advice on clinical trials more or less in parallel. Could this be optimised?

The patient representatives participating in committees and SA are alone to deal with the files (with some help from EMA) it would be more efficient if they could reach out to more patient experts that are under CA (and have an update DoI) to collect info and maybe discuss their opinion.


Thank you!