
aparito
™

Co-creation with Patients: How to develop meaningful Clinical Outcome Assessments (COAs)

Dr Elin Haf Davies PhD
Founder & CEO
Aparito

July 2022

WORLD OrpharDrug Congress USA 2022

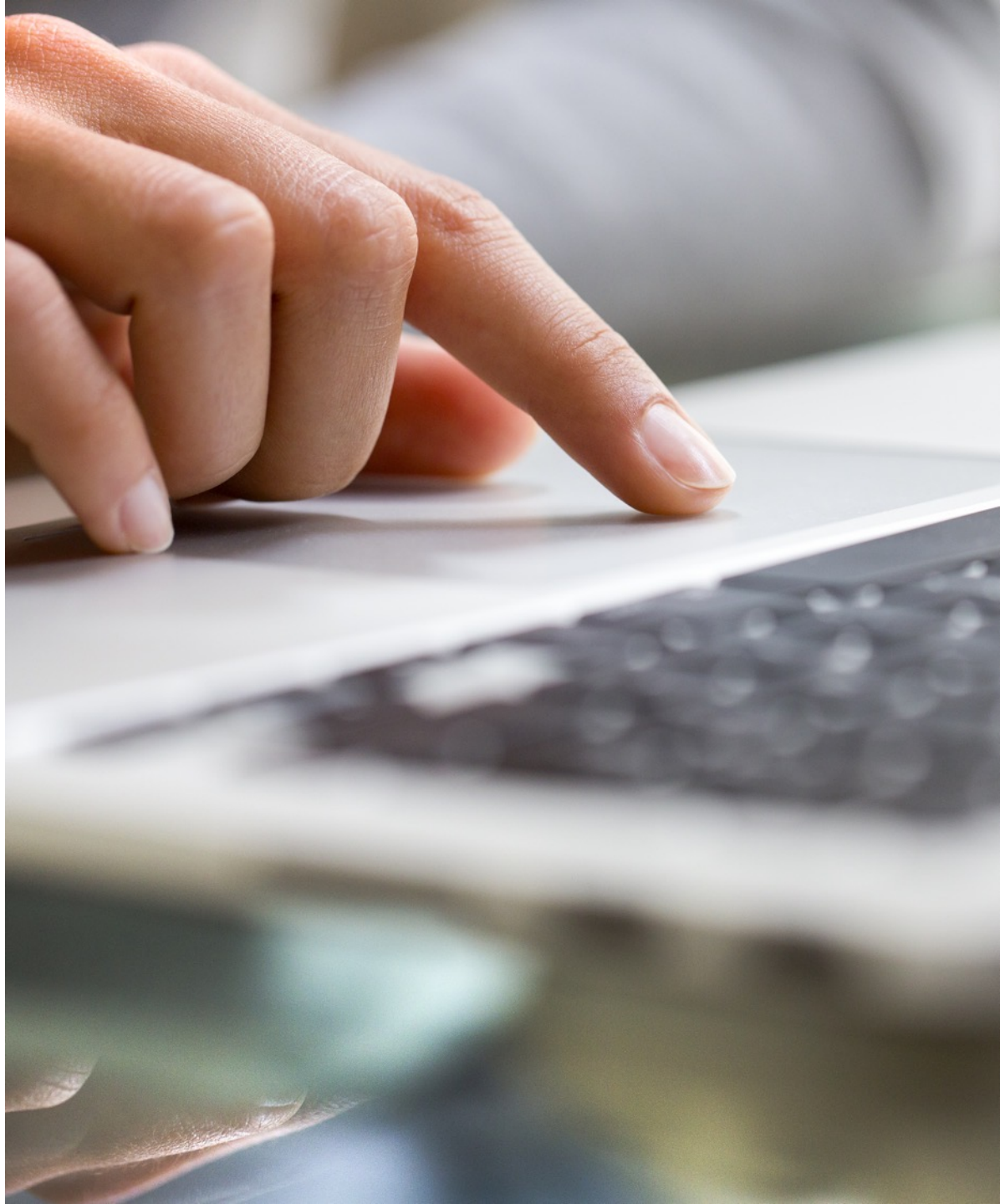
Agenda

Who we are?

Our platform: Atom5™

Our approach to Co-Creation with Rare Diseases

Questions & Discussion



We are Aparito

Patients living with life-limiting diseases need access to clinical trials and innovative treatments.

Aparito provides that access, wherever patients are.

Global Partners

Prevail Therapeutics

LYSOGENE

Recursion

labcorp Drug Development

Kyowa Kirin

Amicus Therapeutics

Takeda

Neurogene

Phoenix Nest INC

What We Do

Aparito brings clinical trials to patients and unlocks real-world data through mobile apps, video assessments & wearable devices.

We provide a patient-centric platform that integrates clinical & regulatory expertise to capture patient data and develop digital endpoints for hybrid and decentralized clinical trials to streamline the drug development process.

ISO/IEC 27001

Our Journey So Far

WORLD OrpharDrug Congress USA 2022

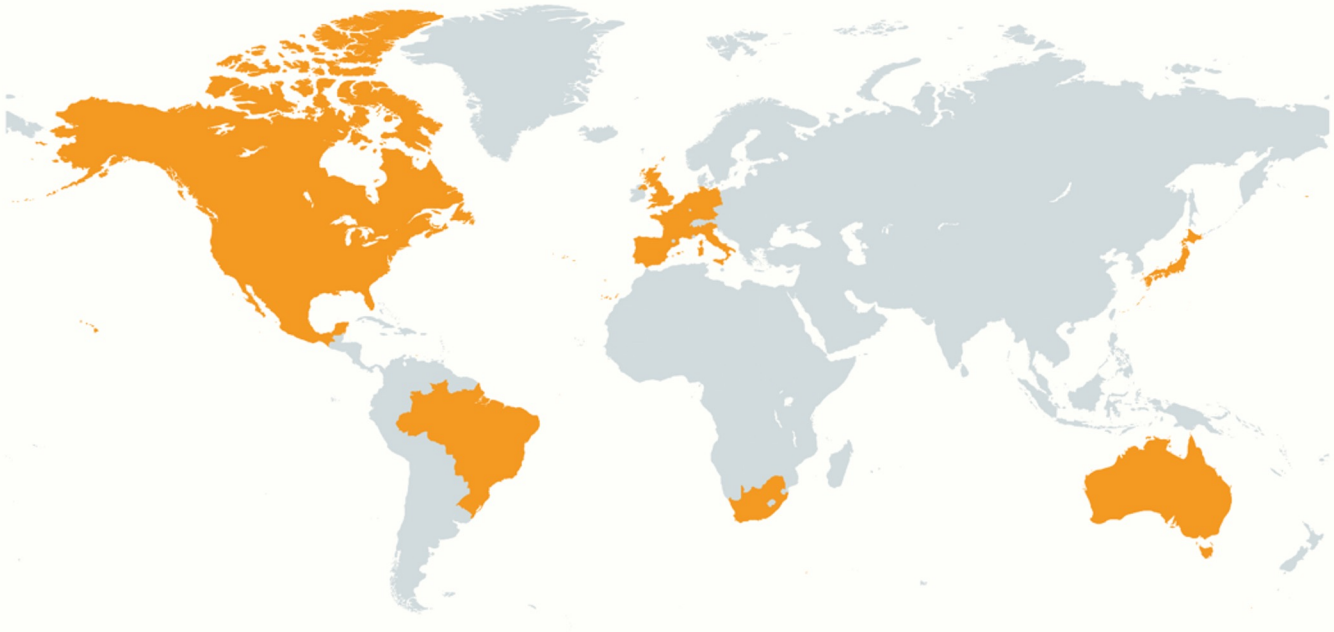
2014	2015	2016	2017	2018	2019	2020	2021	2022
Late Onset Tay-Sachs	Gaucher Disease	Juvenile Idiopathic Arthritis	Narcolepsy	Sanfilippo	Pompe	Parkinson's Disease	CLN5	Osteogenesis Imperfecta
Niemann – Pick C	Duchenne Muscular Dystrophy	Complex Epilepsy				Chronic Pain	Gaucher Disease	Primary Ciliary Dyskinesia
						Bowl & Bladder support tool	XLH	Sanfilippo Syndrome (MPS-IIID)
						Psoriatic Arthritis	Leber Congenital Amaurosis	Long COVID
						Cancer care	Cerebral Cavernous Malformation	Angelman Syndrome

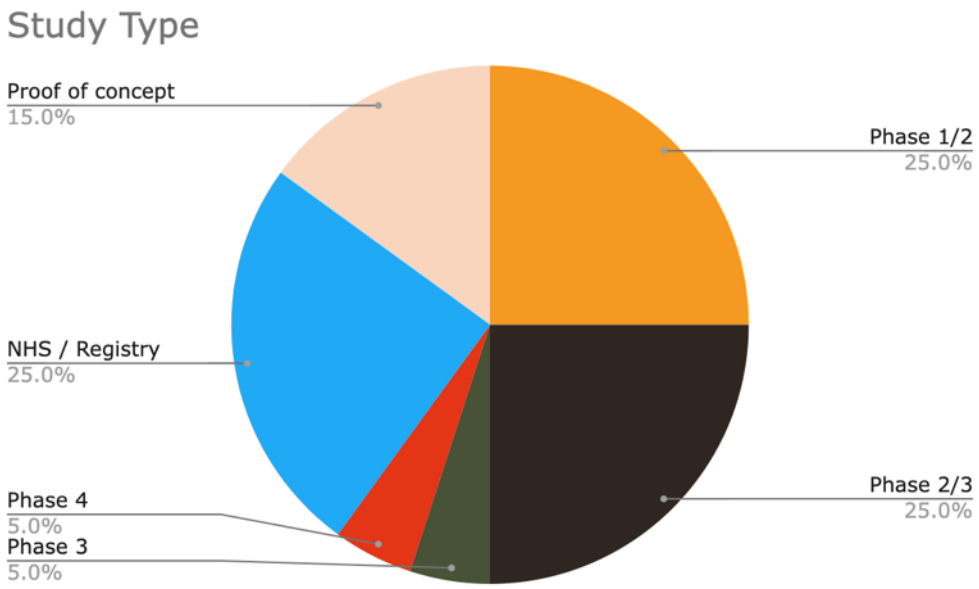
Extensive experience in Rare Diseases

With proven experience in developing solutions for rare disease studies and 50+ publications to date, Aparito expanded the use of its platform so more patients can benefit

EURORDIS Black Pearl AWARDS

Global Reach





Aparito has deployed our technology in 20+ countries to date with 8000 patients enrolled.

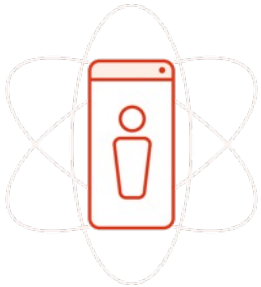
Our Platform:

atom5™

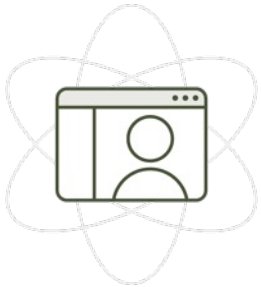
WORLD OrpharDrug Congress USA 2022


atom5™
Powered by
aparito

Atom5™ Features



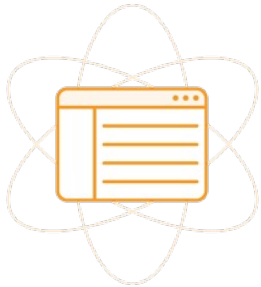
Patient App & Dashboard



Clinical & Sponsor Portal

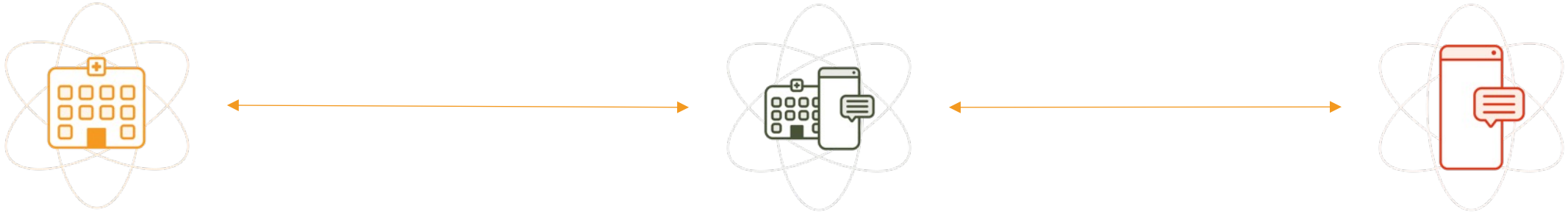
eCOA

Data Analytics



Regulatory Compliance

Digitizing Clinical Trials

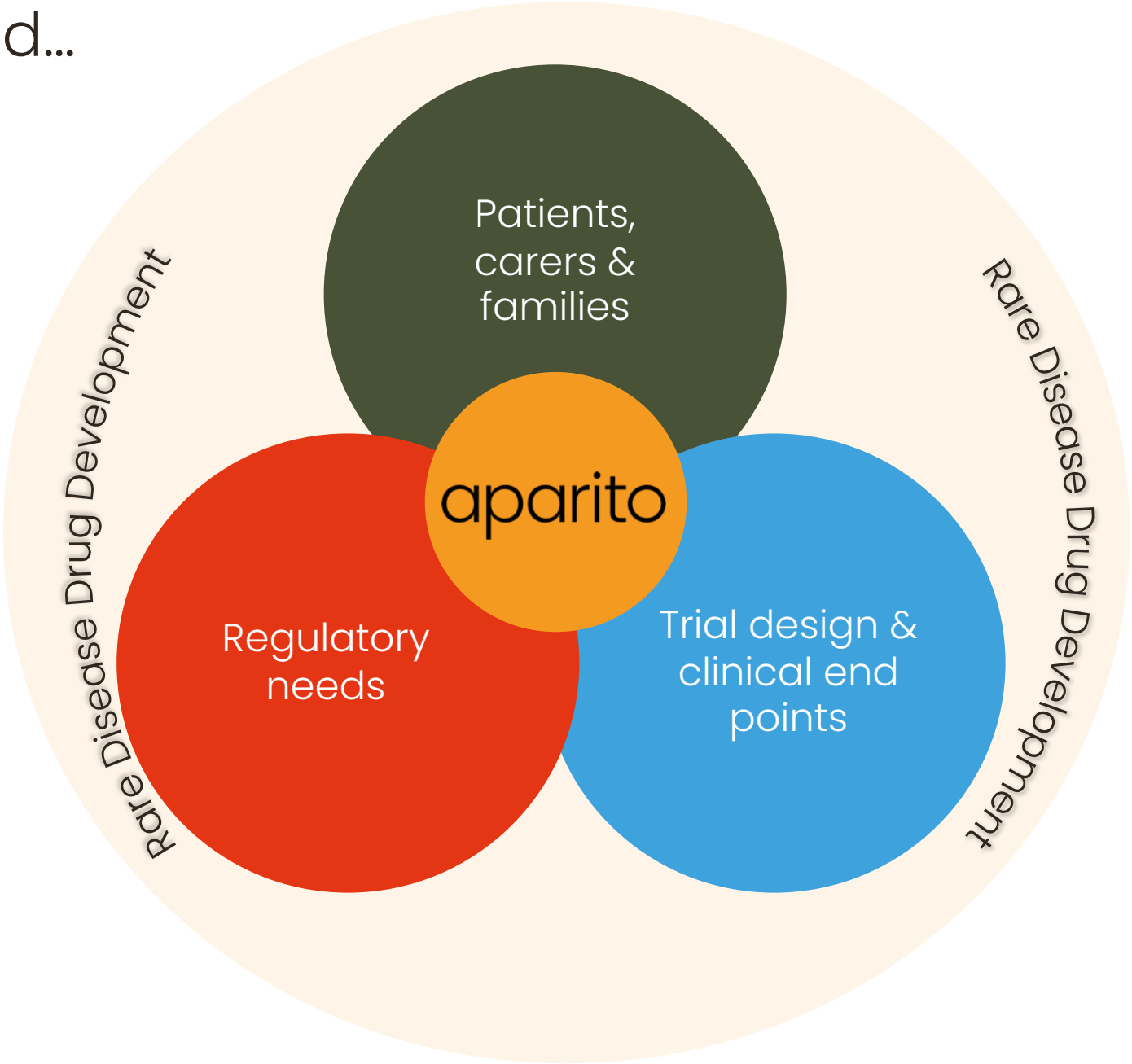


Traditional Trial
The site-based model has limited use of digital data capture and limited data captured outside of scheduled clinic visits.

Hybrid Trial
Hybrid trials *combine* site-based assessments *with* remote data capture to *digitize clinical trials*.

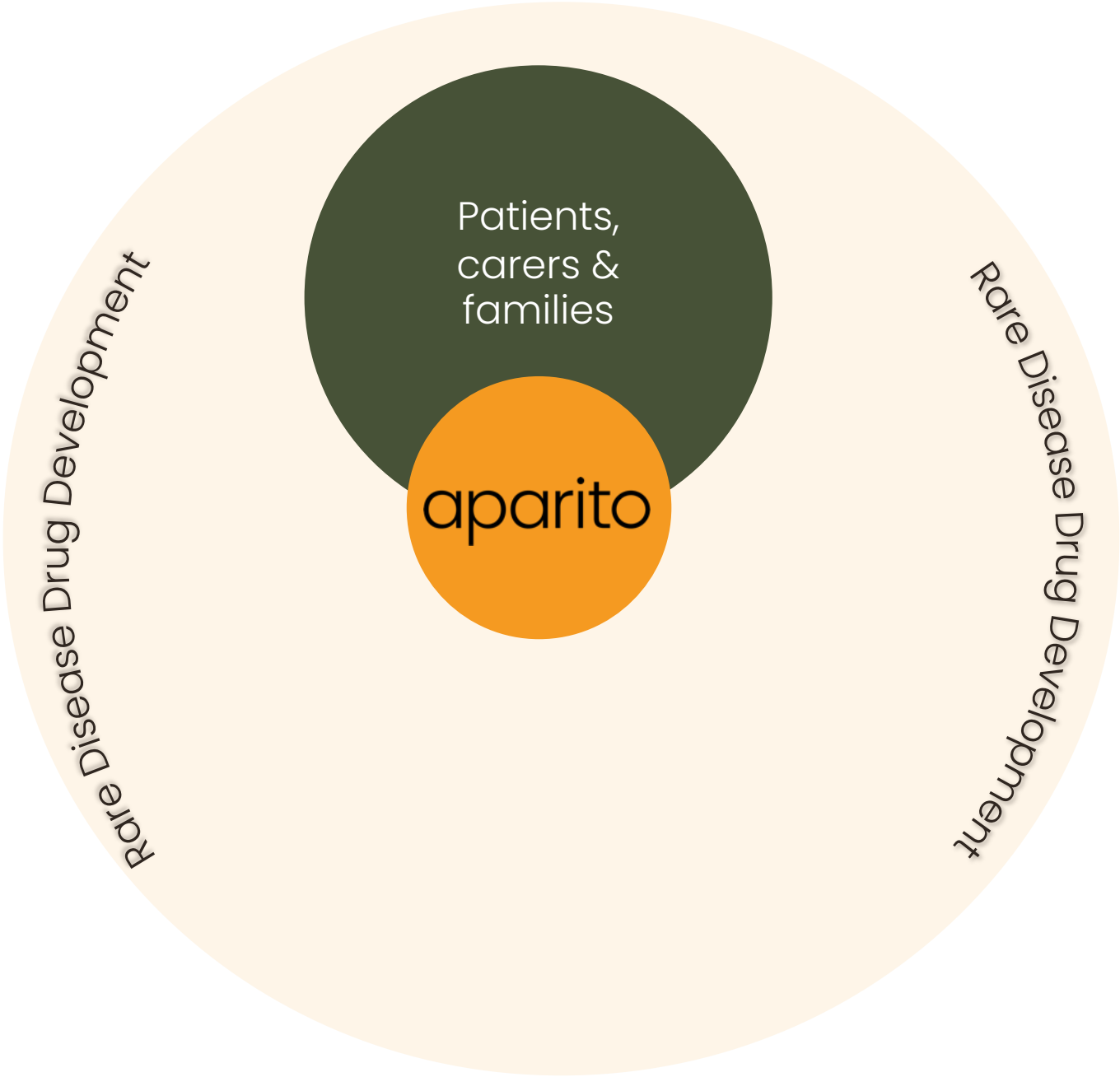
Decentralized Clinical Trial (DCT)
DCT eliminates the traditional site model with all recruitment, consent, onboarding and data capture conducted remotely.

We understand...



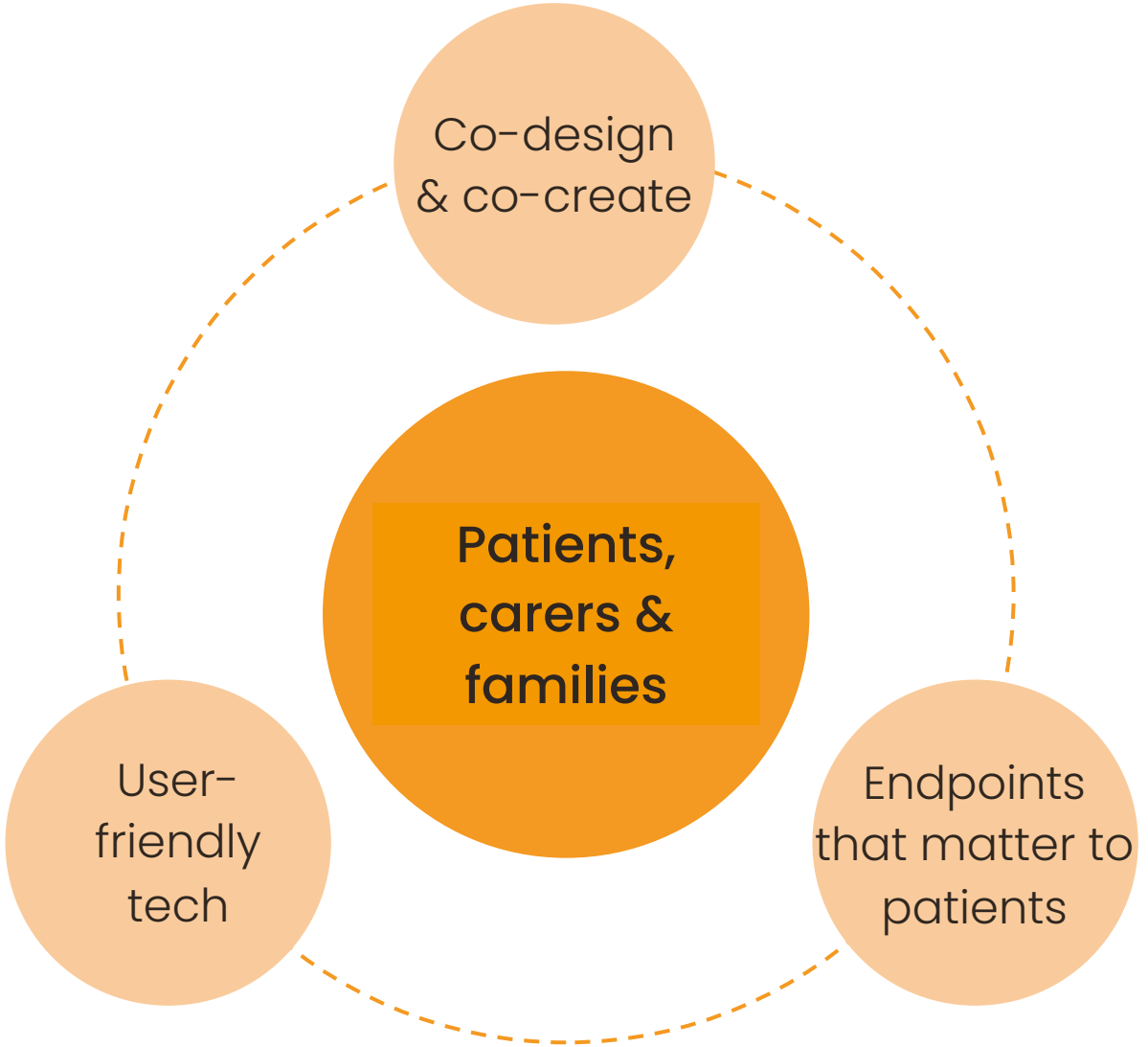
…and translate it to value throughout the study lifecycle

eCOA Endpoint Strategy Consultation	Protocol Development & Study Design	Study start-up & Maintenance	Study Close -out
Early-stage support with input from **Patient Group Accelerator Programme** to guide Clinical Development: - Gap analysis and biomarker/instrument identification - Conceptual & endpoint model development	Protocol review and recommendations for hybrid clinical trials with input from Accelerator: - Assessments & endpoints - eDiary design - Relationships with copyright holders	Patient, site training Independent wearable & video data reviews Data quality monitoring & eCOA oversight	Data interpretation Rapid database lock Guidance on next development phase/lifecycle opportunities
Design & Strategize		Operationalize	Close-out


Patients, carers & families
aparito
Rare Disease Drug Development
Rare Disease Drug Development

Our Patient Advocacy Credentials

Aparito Patient Group Accelerator

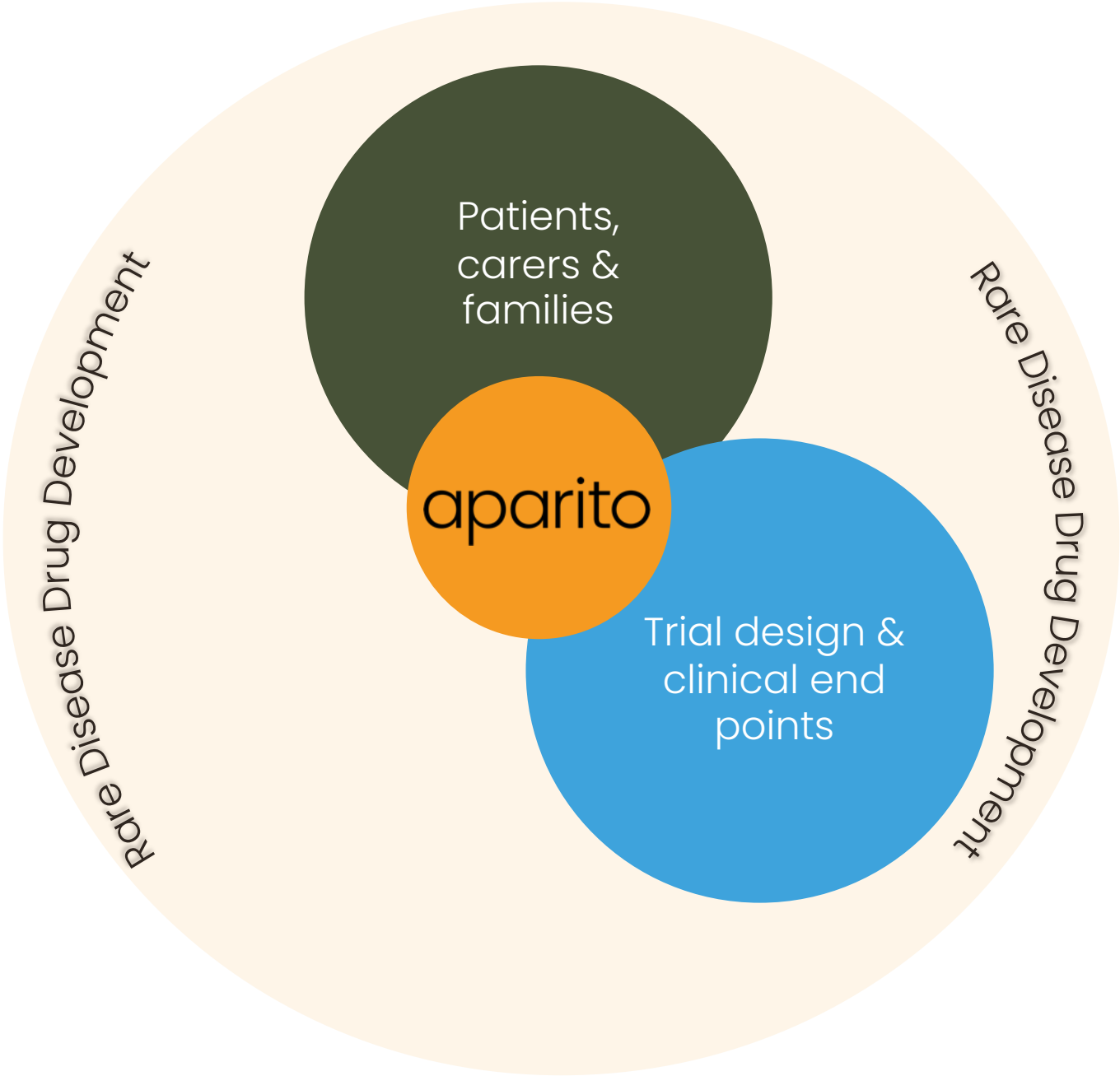


Accelerator Co-creation Approach

Patient Group Accelerator Programme

Patient Needs Assessment	Selection of Outcomes of Interest	Technology Development	Tech Usability & Feasibility Testing	Analysis
Duchenne UK	Measuring transfer stage	Digital platform supporting video capture and AI-based analytics	Prototype testing in small group of patients	Analysis strategy & interpretation
PCD SUPPORT UK	Digital health to support engagement with treatment routines	Mobile app and wearables	Decentralized study with rewards and Gamifications	

- Feasibility, acceptability and useability of the technology
- Exploratory data to support the clinical utility of the technology
- Enable software compliance for future qualification of the technology


Rare Disease Drug Development
Patients, carers & families
aparito
Trial design & clinical end points
Rare Disease Drug Development

Biomarkers & Drug Development

Possibility of Success (POS)
with biomarker

Possibility of Success (POS)
without biomarker

"Use of biomarkers in the stratification of patients improves the POS in all phases; most significant in Phases 1 and 2."

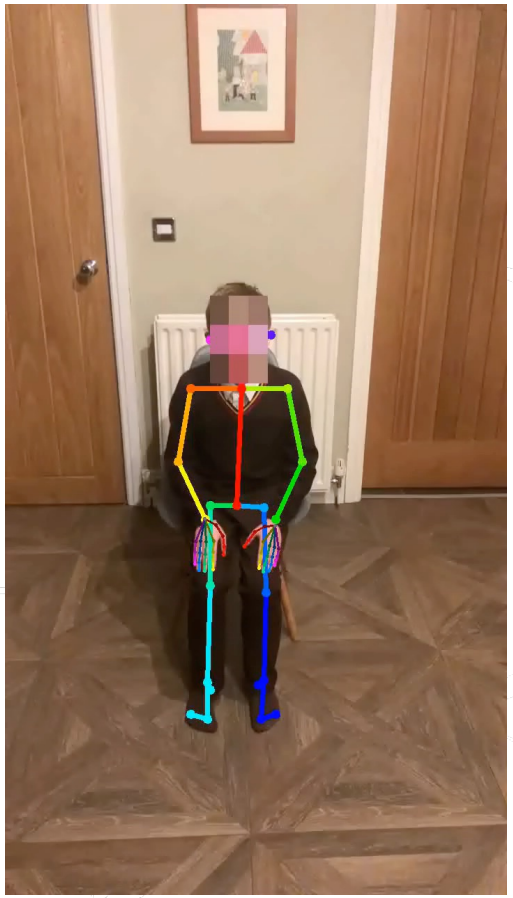
Wong, C. H., Siah, K. W., & Lo, A. W. (2019). Estimation of clinical trial success rates and related parameters. Biostatistics, 20(2), 273-286.

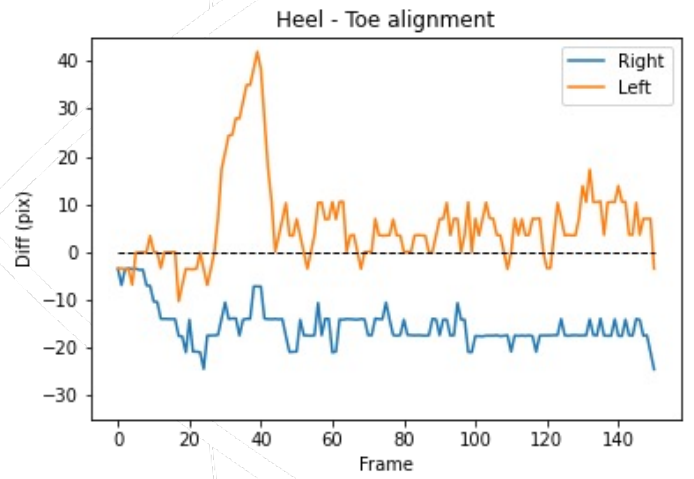
Digital endpoints driven by patient needs

Use of 6-min walk test in Duchenne muscular dystrophy as primary endpoint in drug clinical trials, excluded patients who were no longer able to walk.

Outcome measures that capture the transition from the ambulatory to the non-ambulatory stage at home was needed.

A well-planned COA strategy in rare disease and pediatric trials is critical to support the selection and interpretation of COA endpoints





Example: DMDhome & Video Analysis

Developed to identify objective outcome measures to capture the transition from the ambulatory to the non-ambulatory stage in DMD patients using Atom5™.

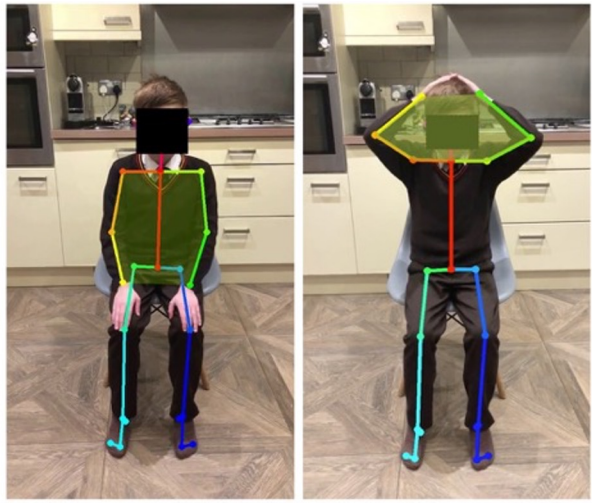
Selected tasks captured with DMDhome app:
- Walking
- Sit-to-stand
- Hands-to-head

Video recordings analyzed using OpenPose, a pose estimation software to detect points on human body, face, hands and feet in videos

Can detect multiple people, or can restrict to a specific number

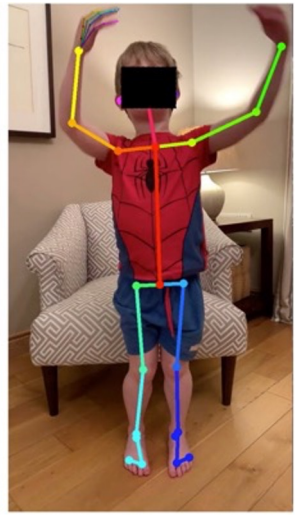
Identifies 25 points on the body

Hands-to-head parameters



A B

Sit-to-stand parameters

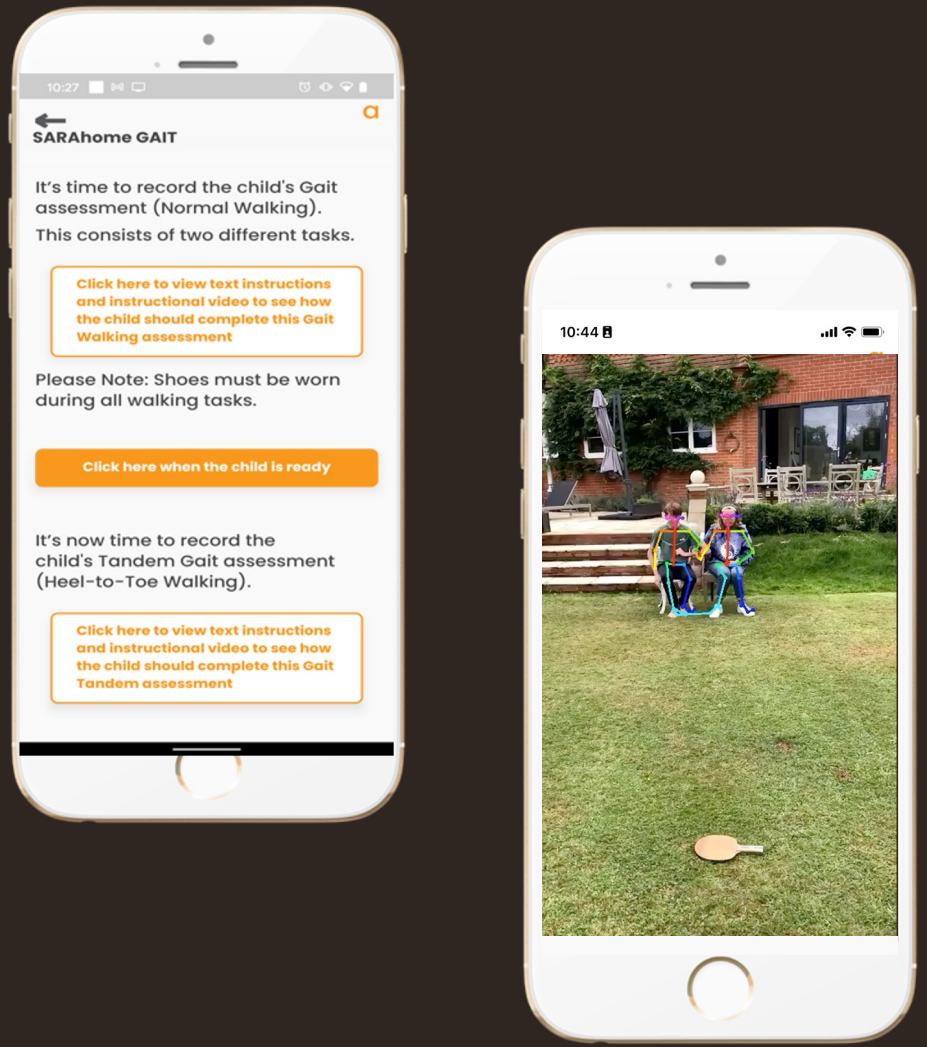


Body points captured by OpenPose show the area defined by shoulders, elbows and wrists when patient hands are in the resting position (A) and when hands are on the patient's head (B).

GARDIAN Registry

Our vision for GARDIAN is to:

- Enable patients to enroll in the registry wherever they live in the world
- Get patients and caregivers to add their own data to the nGD-specific patient-reported outcome (PRO) and caregiver-reported outcome (ObsRO)



vTUG

Atom5™ Unique Offerings

SARA^{home}

The SARA tests have been digitized on the Atom5™ platform as SARA^{home}

The SARA^{home} and the conventional SARA are highly correlated.

SARA^{home} is completely video-based without the need for examiner

vTUG

The traditional TUG test has been digitized on the Atom5™ platform and uses video capture and pattern recognition to enable objective, sensitive high frequency assessments.

PROVide

Novel exploratory outcome measures for MPS IIIA running in parallel (sub-study) to AAV10-SGSH intracranial gene therapy trial

Location: Global

Condition: MPS IIIA (San Filippo Syndrome)

Duration: Ongoing

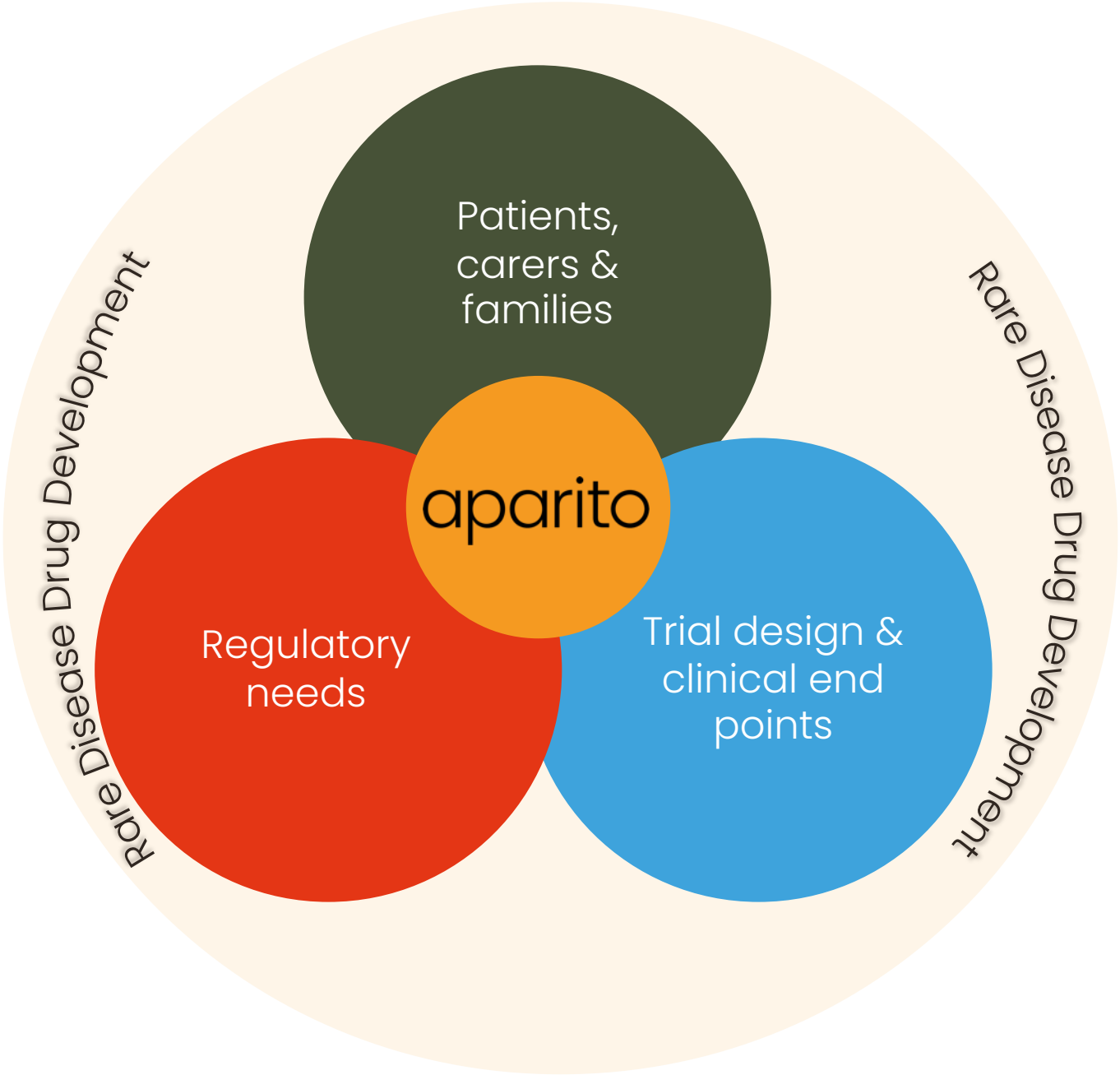
Aim of this study

To assess the value of **video assessments** to capture disease stabilization and improvement in MPS IIIA patients during the P4-SAF-302 trial.

To monitor the onset and evolution of disease hallmarks of MPS IIIA patients using video assessment.

To explore the observations of caregivers of MPS IIIA patients during the P4-SAF-302 trial.

To utilize video assessment for capturing any new abilities that MPS IIIA patients gain during the P4-SAF-302 trial.


Patients, carers & families
aparito
Regulatory needs
Trial design & clinical end points
Rare Disease Drug Development
Rare Disease Drug Development

Adaptive study designs: Support from regulators

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

12 May 2014
EMA/44410/2014

Gaucher disease
A strategic collaborative approach from EMA and FDA

Draft presented at EMA Workshop with European Working Group on Gaucher disease (EWGGD) and European Gaucher Alliance (EGA)	October 2011
Comments from FDA, Health Canada and Japan	April 2012
Comments from Paediatric Review Committee (FDA)	July 2012
Joint workshop with FDA to consult industry, experts and patient organisations	17-18 September 2012
Comments from Scientific Advice Working Party (EMA)	September 2012
Comments from FDA	July 2013
Adopted by Scientific Advice and Committee for Human Medicinal Product (EMA) for release for consultation	March 2014
Adopted by FDA and Paediatric Committee (EMA) for release for consultation	May 2014
Start of public consultation	14 May 2014
End of consultation (deadline for comments)	31 August 2014

Comments should be provided using this template. The completed comments form should be sent to extrapolation@ema.europa.eu

EMA

"Comprehensive knowledge of a disease can help design and conduct **adequate and well-controlled clinical trials** of adequate duration with **clinically meaningful endpoints**…"

Clinically meaningful and relevant endpoints require **informative natural history studies and patient input**

– Patient-focused drug development (PFDD) meetings

Pediatric Rare Diseases — A Collaborative Approach for Drug Development Using Gaucher Disease as a Model
Guidance for Industry

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft guidance. Submit electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document, contact Hong Vu at 301-796-7401.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

December 2017
Rare Diseases

16252414dft.docx
11/28/17

FDA

Regulatory Compliance

The Atom5™ global platform operates under ISO143485 QMS and ISO/IEC 27001 ISMS accreditations and is FDA 21 CFR Part 11 and GDPR compliant.

Thank you!

Dr Elin Haf Davies PhD
Founder & CEO
Aparito

elin@aparito.com

Visit us at booth #426!

WORLD OrpharDrug Congress USA 2022


aparito
™