Mobile data collection to improve drug development

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Two Major Trends in Healthcare

« the perfect storm »

Patient-Centricity Participatory Engaged Increased Expectation	Digital Health Internet of Things Precision Medicine

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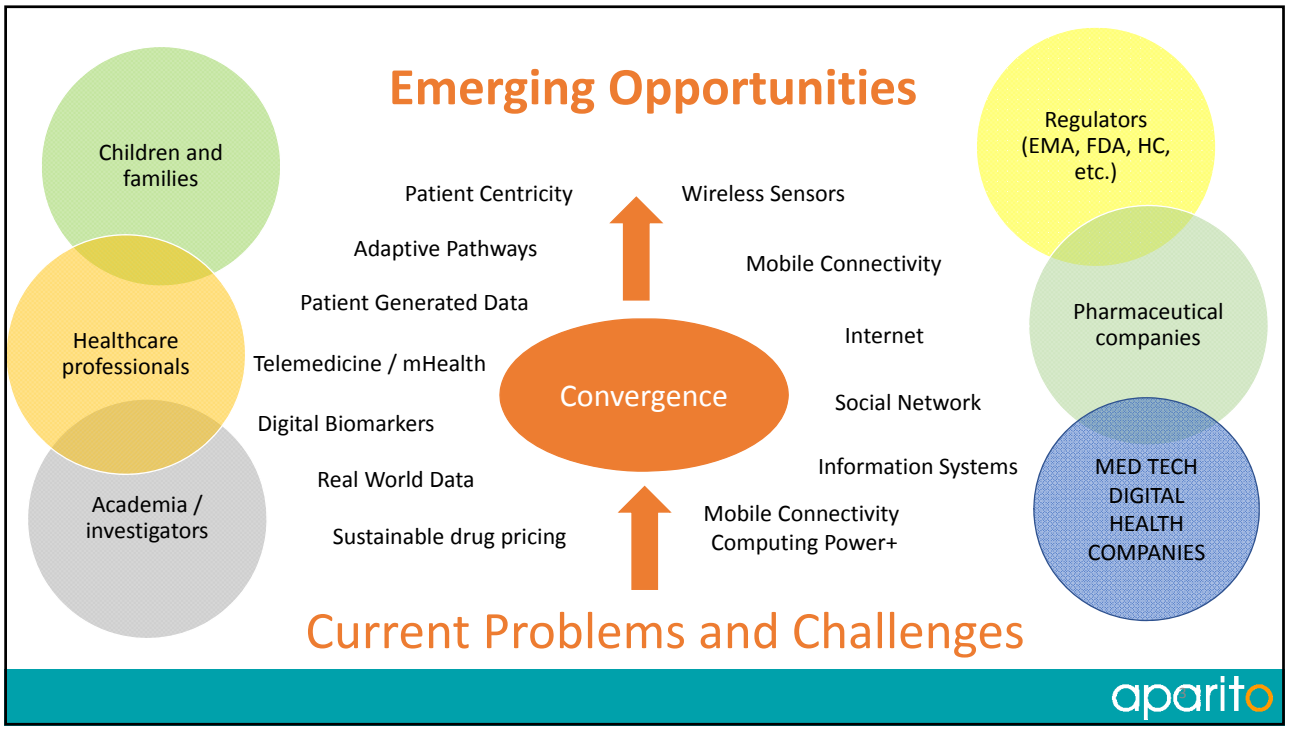
Emerging Opportunities
Children and families
Healthcare professionals
Academia / investigators
Patient Centricity
Adaptive Pathways
Patient Generated Data
Telemedicine / mHealth
Digital Biomarkers
Real World Data
Sustainable drug pricing
Convergence
Wireless Sensors
Mobile Connectivity
Internet
Social Network
Information Systems
Mobile Connectivity
Computing Power+
Regulators (EMA, FDA, HC, etc.)
Pharmaceutical companies
MED TECH DIGITAL HEALTH COMPANIES
Current Problems and Challenges
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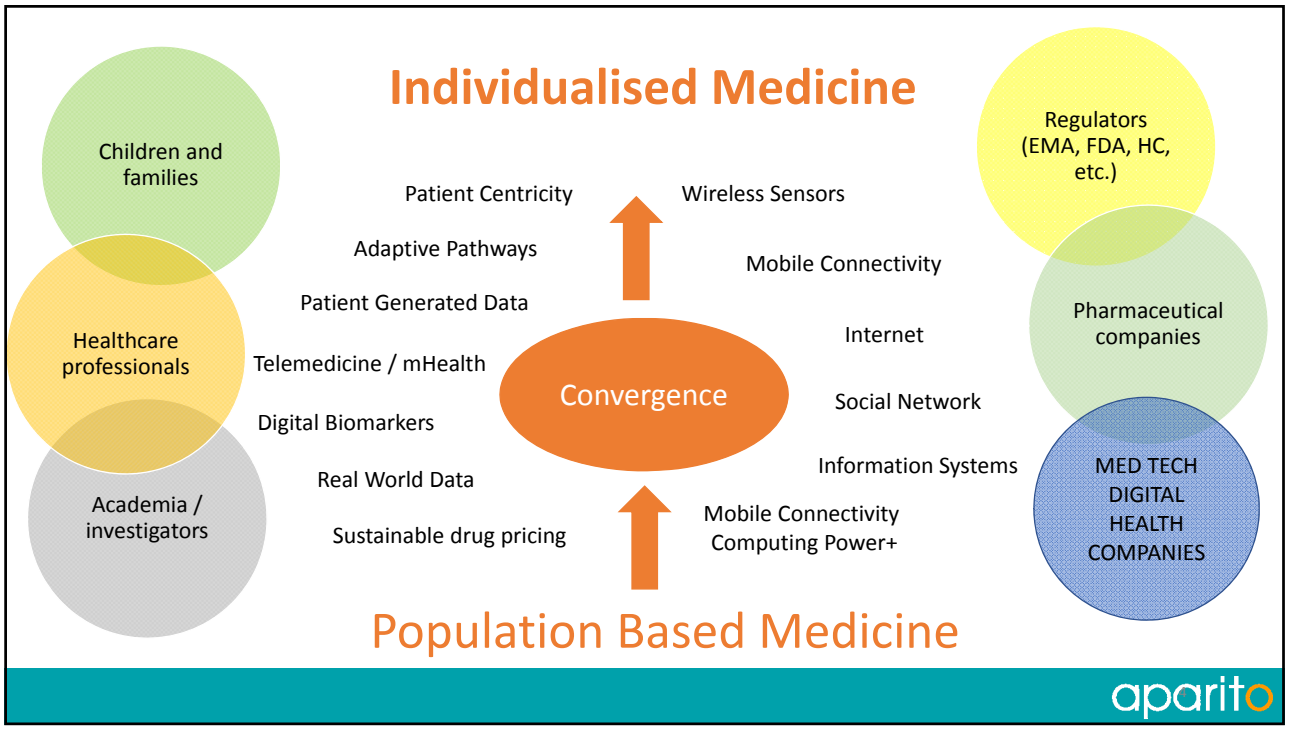
Individualised Medicine
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MED TECH DIGITAL HEALTH COMPANIES
Population Based Medicine
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The Problem

Current challenges to recruit and retain trial participants.

Clinical study protocol complexity:
a major cost driver

A Typical Phase III Protocol	2001 - 2005	2011-2015
Total Number of Endpoints	7	13
Total Number of Eligibility Criteria	31	50
Total Number of Procedures	110	187
Total Number of Procedures per visit	10	13
Total Number of Countries	5	10

Source Tufts CSDD

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The Problem

Current challenges to recruit and retain trial participants.

Participation Burden &
Missed Engagement

What did you least like about your participation experience? (Top 5 mentions)	Percent of Total
Not knowing whether I was getting the investigational treatment	30%
Location of the research center	22%
Study visits were too time consuming	19%
Compensation was not enough given the demands of the study	16%
Study procedures were too cumbersome	15%

Source Tufts CSDD

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The Problem

Current challenges to recruit and retain trial participants.

Staff availability

Clinical Site Capacity

Limited space / clinical room

Multiple trial burden

High costs <100% overheads for industry>

Source Tufts CSDD

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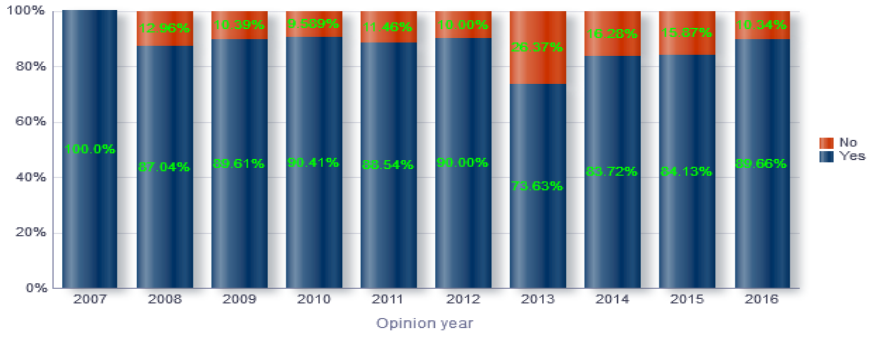
The Problem



Deferrals

7 years from approval of PIPs in EU* (similar lag in US) to authorisation:

- **Only 17%** of medicines authorised for adults completed required paediatric trials
- Studies to be completed after initial authorisation were <u>unlikely to be completed</u> as compared to those to be completed before authorisation

** (Ollivier et al, Nov 2018, Br J Clin Pharmacol)*

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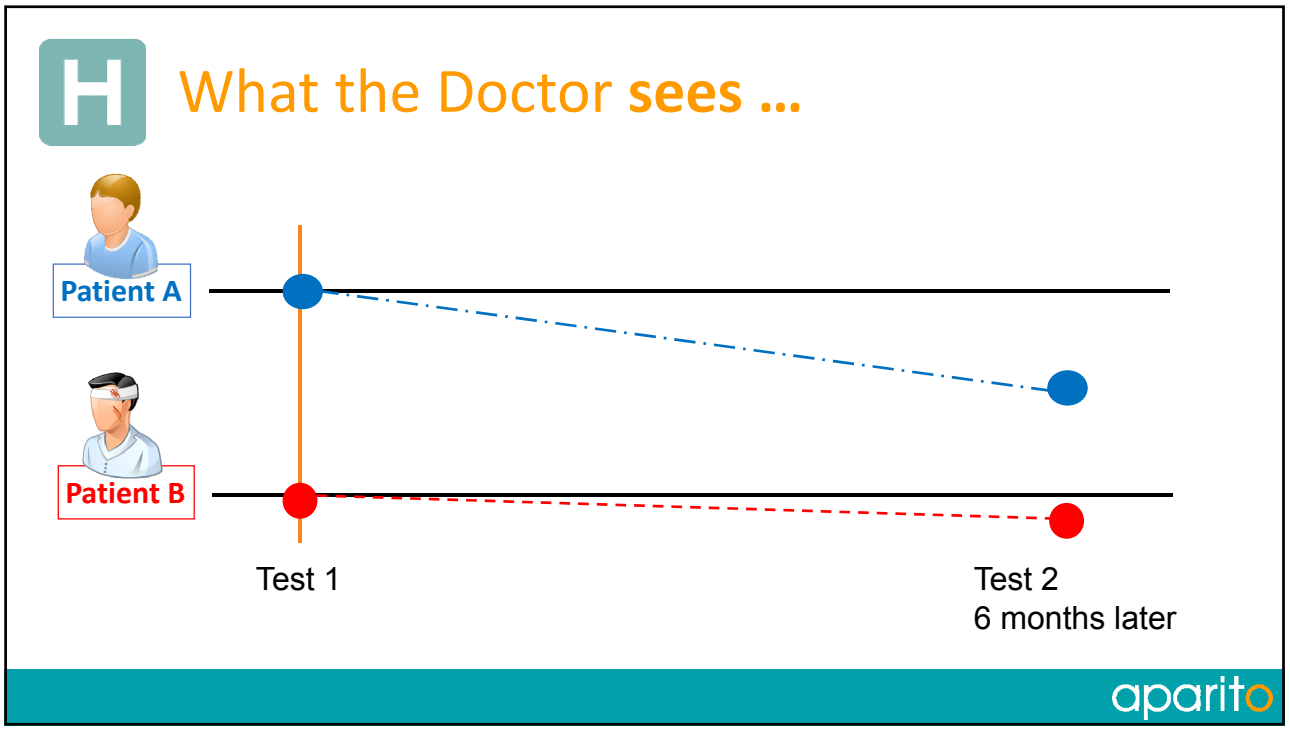
What the Doctor sees …
Patient A
Patient B
Test 1
Test 2
6 months later
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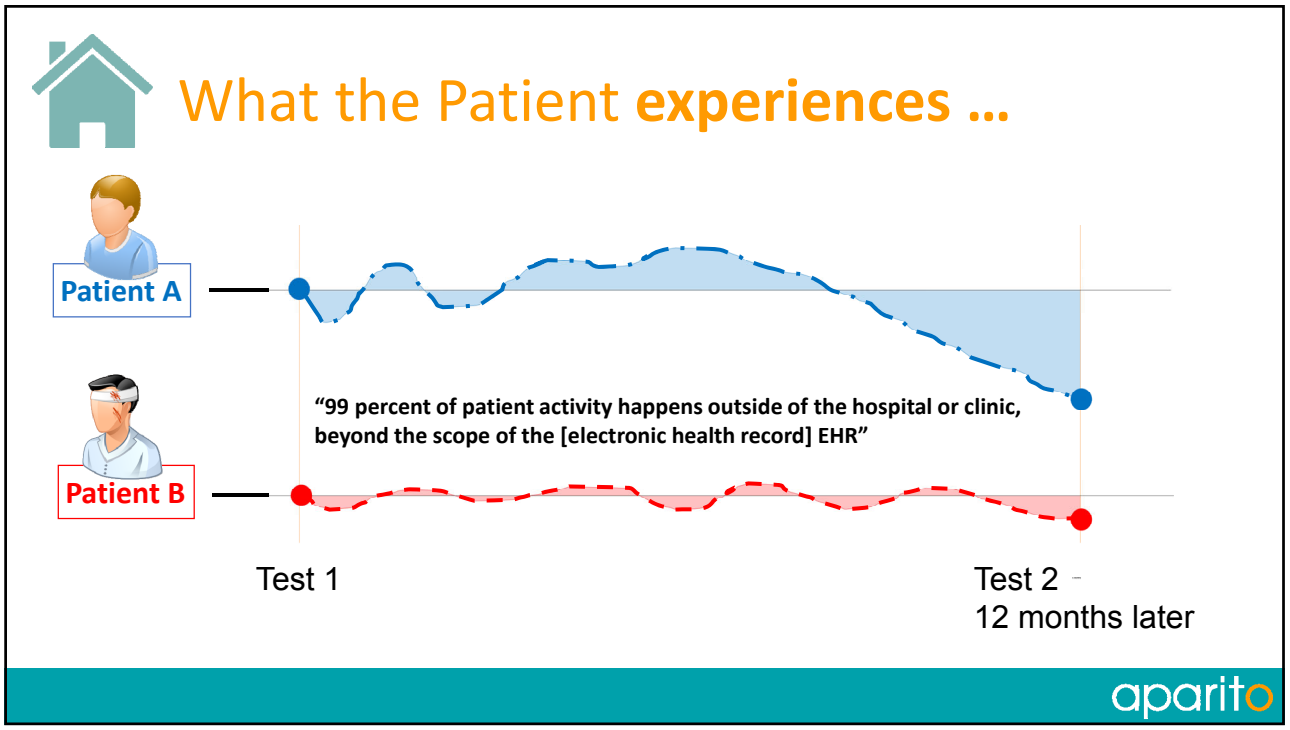
What the Patient experiences …
Patient A
Patient B
"99 percent of patient activity happens outside of the hospital or clinic, beyond the scope of the [electronic health record] EHR"
Test 1
Test 2
12 months later
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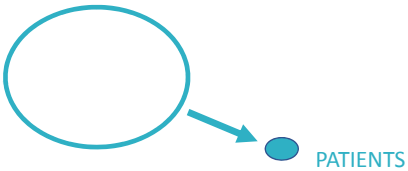
Patient Centricity

« Hot air and hype or a happening reality »

CIRCLE OF TRUST



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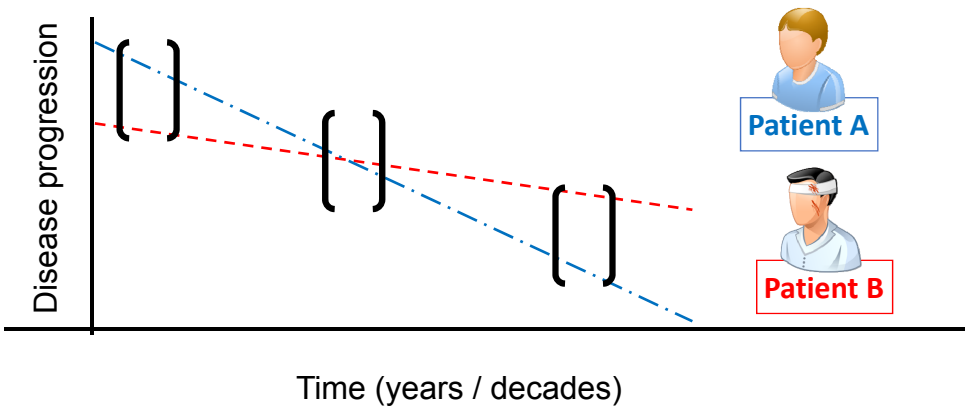
Heterogeneous disease progression – added complexity



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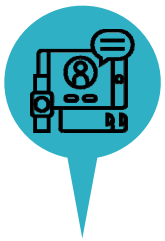
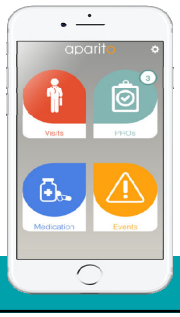


Patient insight and engagement



Patient Generated Data

Patient-generated data (PGD): created, recorded, or gathered by or from patients

- health related events / symptoms (videos, photos, voice, text)
- medication adherence
- biometric data (wearable devices)
- ePatient Reported Outcomes (PROs)

Patient-generated data (PGD) ≠ clinical settings:

- Patients, not providers, are responsible for capturing or recording these data.
- Patients decide what data to share, and with which health care providers / researcher to do so.

(e.g. physical activity using wearable devices, medication adherence and ePRO using a mobile app.)

PGD most easily captured digitally in today's world.
Digital Biomarker becoming an important concept.

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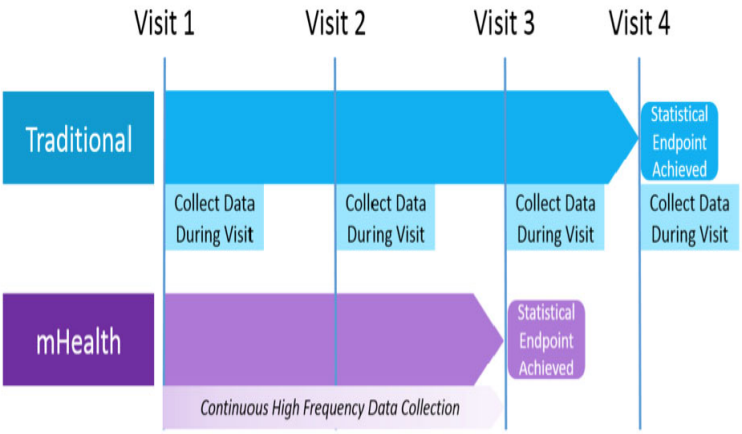
New insight

Improving timelines



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Do all data have the same value?

Study Type	Cost	Sample Size	Data quality	Data type
Clinical Trials	Very Expensive	Small: optmisied to show efficacy	Very high	Clinical Static Episodic Predefined
Natural History / RWD	Significant	Significant: e.g. 10% of all patients types	Good	Clinical Static Episodic Opportunistic
Patient Registries	Lower	Variable (low)	Variable / Low	Clinical Static Episodic Opportunistic

Patient Generated Date

Digital Biomarkers / Endpoints

Real World Data

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Clinical Trial Models

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Ambition for Patient Generated Data

Design to overcome increasing clinical demands

- ✓ Bring a different kind of patient insight, participation and engagement
- ✓ Improve disease understanding through PGD and ML/AI
- ✓ Increases the chance of regulatory drug approvals
- ✓ Digital solutions supporting BYOD to enhance patient centricity
- ✓ Empower the patient - reduce hospital visits and increase QoL

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The Aparito solution

Designed to overcome clinical & regulatory roadblocks

- Patient Generated Outcome Data
- Continuous Data
- Digitalised Monitoring Process
- Remote Disease Monitoring
- Real Time Data
- Real Time Flags
- Passive Data Capture
- Paperless Data Capture
- Medication Prompts

✓ Vastly improved understanding of diseases
✓ Increased chance of regulatory approval
✓ Better, cheaper & faster drug development
✓ Higher patient empowerment & quality of life
✓ More participants & engagement
✓ Lives Saved & Improved

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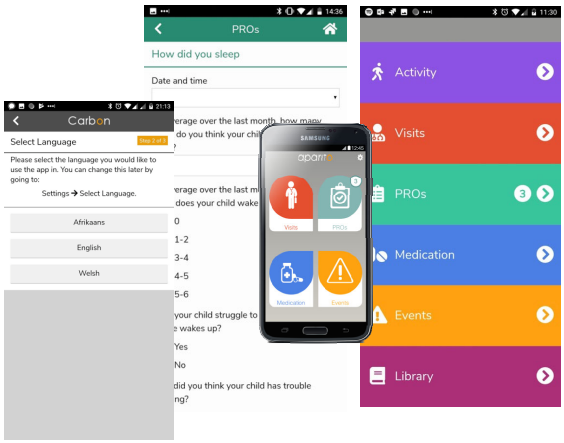
Sophisticated and customized

- Clinic sets up and launches data collection via the web based system
- Patients can be on-boarded remotely via a simple and secure QR code system
- Wearables synchronize directly with the phone app - No need to use multiple apps



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Current & Completed Trials

Achieving real-world experience in rare diseases / paediatrics

NIEMANN PICK C
- ACTELION
- Great Ormond Street Hospital for Children NHS Foundation Trust
- Central Manchester University Hospitals NHS Foundation Trust

JUVENILE IDIOPATHIC ARTHRITIS
- The Newcastle Upon Tyne Hospitals NHS Foundation Trust

DUCHENNE MUSCULAR DYSTROPHY
- DUCHENNE UK
- The Newcastle Upon Tyne Hospitals NHS Foundation Trust
- Great Ormond Street Hospital for Children NHS Foundation Trust

GAUCHER DISEASE
- Shire genzyme
- Central Manchester University Hospitals NHS Foundation Trust
- MediSoft

LATE ONSET TAY SACHS
- NIH National Institutes of Health

CHILDOOD EPILEPSY
- RED CROSS WAR MEMORIAL Children's

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On-going activities

MPS IIIA

DUCHENNE MUSCULAR DYSTROPHY

HERCULES.

EURORDIS Rare Diseases Black Pearl

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

20 September 2018
EMA/CHMP/SAWP/527447/2018
Product Development Scientific Support Department

Draft qualification opinion on stride velocity 95th centile as a secondary endpoint in Duchenne Muscular Dystrophy measured by a valid and suitable wearable device*

International Gaucher Alliance
@IGAgaucher

We're pleased to announce the launch of Phase 1 of the development of a Global Patient Registry for neuronopathic Gaucher Disease (nGD) with @aparitohealth, supported by @pfizer, Oxyrane & Prevail Therapeutics. gaucheralliance.org/gb/news_events...

nGD

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Unique opportunity

✓ Improved disease understanding

✓ Better patient outcomes

✓ Connection to patients

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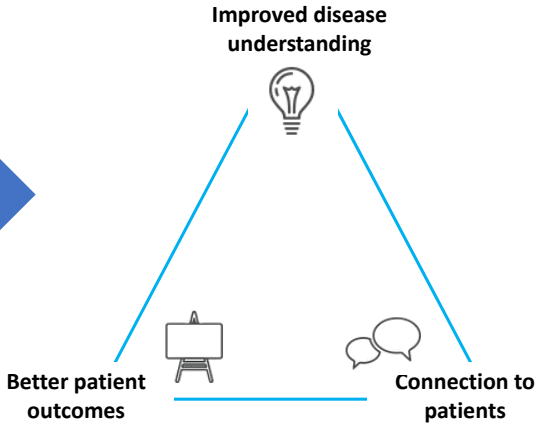
Digital Health: A Unique Solution for a Unique Challenge

Wearables and Disease-specific mobile apps

Real-time monitoring

Outside of the hospital



Adelphi COMMUNICATIONS

Key Enablers and Anticipated Challenges in the EU

Key Enablers

- **Patient Voice**
- Regulatory bodies initiatives
- Operational Feasibility
- Central Procedure for CTA
- IMI context, US TransCelerate
- Digital Health Technologies,
- Uptake of Telemedicine

Anticipated Challenges

- **Data Privacy**
- ICH/GCP (e.g. PI/sub-PI responsibility)
- Lack of EU legal framework for Telemedicine
- No EU central Ethics Committees
- Safety monitoring and regulatory acceptance

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Quality and Validation

Reality

- Patient safety can't be compromised for convenience / cost
- Regulatory bodies (EMA/FDA) are eager to embrace
- Notified Bodies struggling with demand (skill)
- Payers are struggling to calculate the value (cost / pricing)

Risks

- Regulatory science can't keep up with innovation
- Self-regulation not enough across the board
- Hype overrides reality = doom of disillusionment
- Ethical principles don't apply to the tech industry in the same way as it does in health
- Data Privacy at risk for new business models

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Expected Benefits of merging Patient Centricity and Digital Health

- Clinical trial designed around the patient, during which all/ most of assessments are organized at home/ within patient community care using DH
- Potential variant to the full remote approach best fitting to specific drugs (e.g. repurposed) and some disease (e.g. rare disease)
- Main Direct Benefits
  - Better patient centric study design
  - Improved patient access to studies incl. diverse population
  - Rapid recruitment
  - Improved retention
  - Greater real world setting insight
  - Cost saving

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Operational Challenges

What are the issues with supporting patients at home?

- Wearables are easily lost or damaged. This creates logistical and cost challenges
- Wearables will need to be re-synchronized for a number of reasons
- Provision needs to be made for lost or damaged devices
- Supporting patients via conventional, unattended channels is inappropriate
- Not all patients are *"tech savvy"* (patients not users)
- Maintaining high levels of patient confidentiality requires clinical site co-operation

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Conclusion

1. **Patient-generated data (PGD)** and **Digital Biomarkers** offer a new opportunity to support the **communication and engagement** and better understand disease

2. Endpoints designed **with** patients ensure usability / feasibility / acceptability

3. Simple to use, pre-configured technology supports patient engagement

4. Healthcare professional buy-in supports "Patient Activation"

5. The regulatory / compliance landscape is not likely to change – **embrace it!**

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Thank you for your attention

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This product is a CE approved Class 1 Medical Device

CE

Compliant with the EU Medical Device Directive (MDD) 93/42/EEC and meets the essential requirements as a non-measuring device according to EU legislation.