# The Convergence of Technology in Life Sciences and the impact on Drug Discovery and Development

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10 YEARS OF BREAKTHROUGHS A HEALTHIER FUTURE

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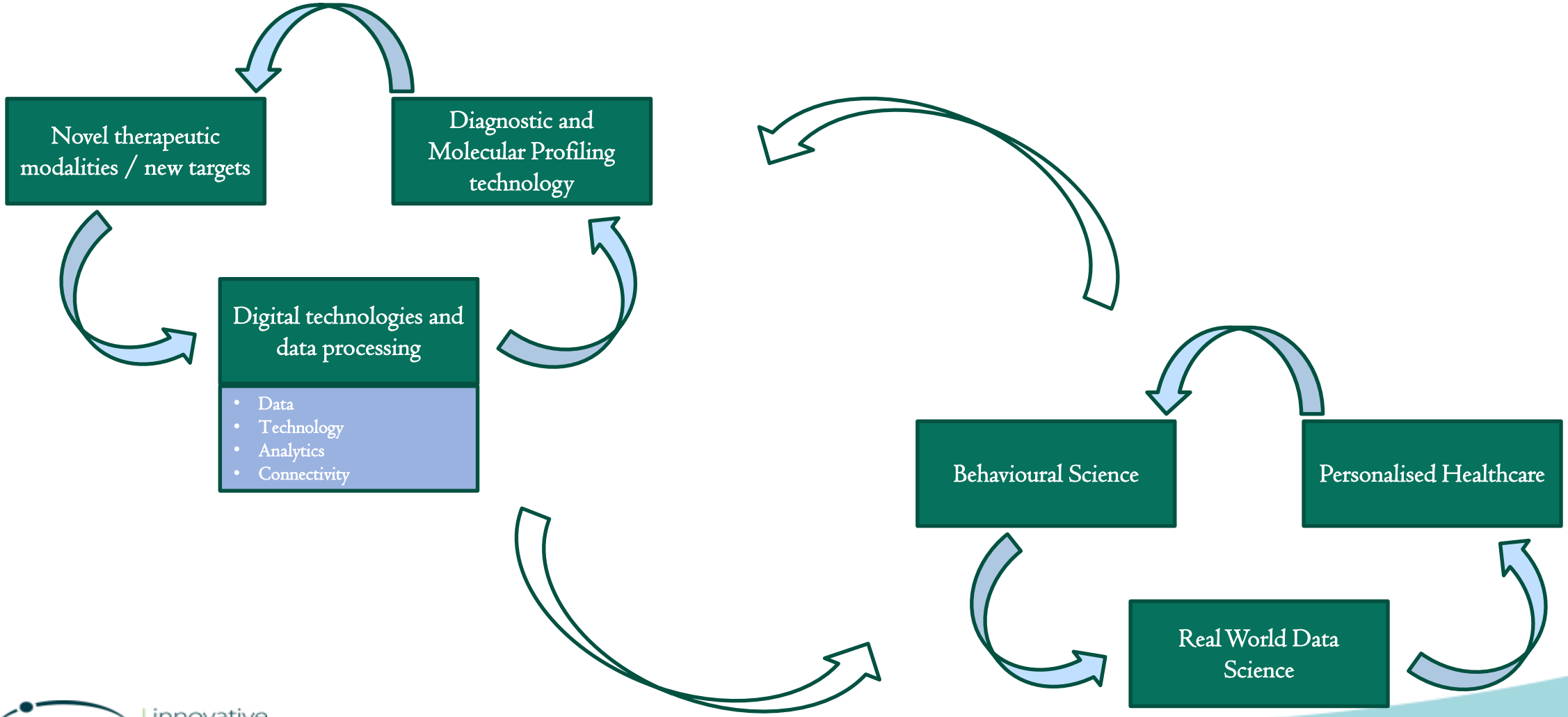
# Interdependent evolution of technology and science

*Faster pace of progress and greater interdependency*

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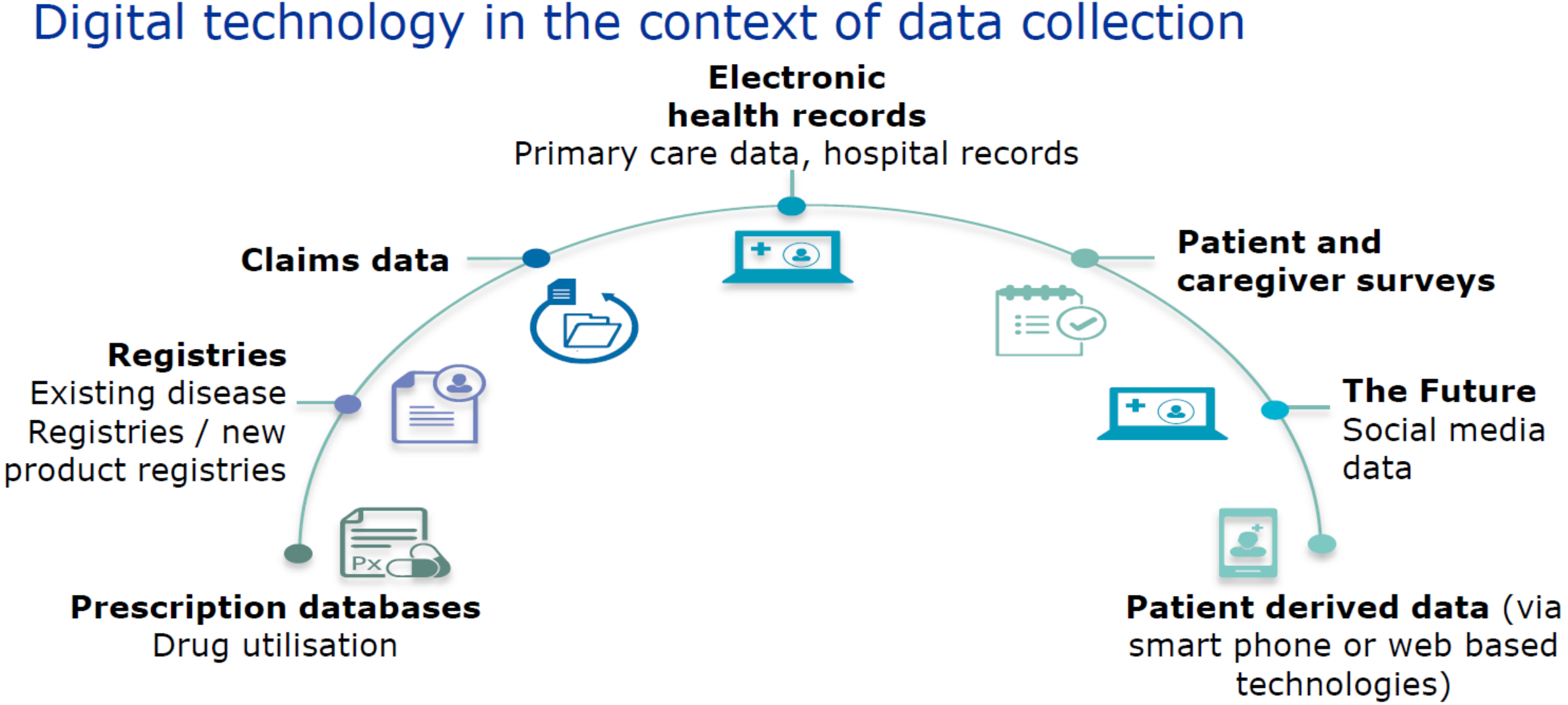
# Digital Technology in the Context of Data Collection




With thanks to M. Berntgen, EMA

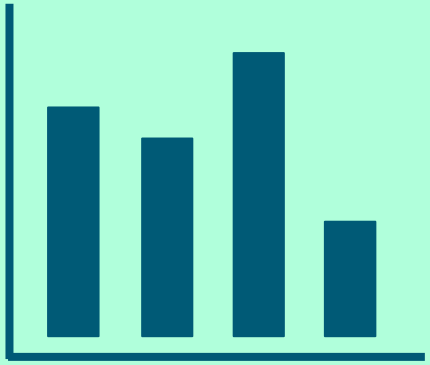
# Personalised Healthcare 2.0 at Roche

## Access meaningful data at scale

- Diagnostic / molecular data
- Clinical trial data
- Real-world data

## Create insights

Advanced analytics of integrated data



FOUNDATION MEDICINE

Roche

FLATIRON

## Generate value

- Drive more effective and efficient R&D
- Improve access & personalised patient care

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# Development of innovative medicines often requires innovation of the entire ecosystem

- At the outset of a development program, particularly in diseases with significant unmet need, much needs to be reviewed:
  - Methods of diagnosis
  - Development «Tool Box»
    - Conceptualization of disease
    - Current SOC and variations therein
    - Biomarkers and other stratification tools
    - Primary and secondary endpoints and methods of assessment
  - Methods of conducting clinical trials
  - Disease management tools
- Innovative technologies are applied to create more patient-centric, efficient and effective development programs

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# Autism Spectrum Disorders

*Core and co-morbid symptoms*

## Incidence

- 1 in 68 births

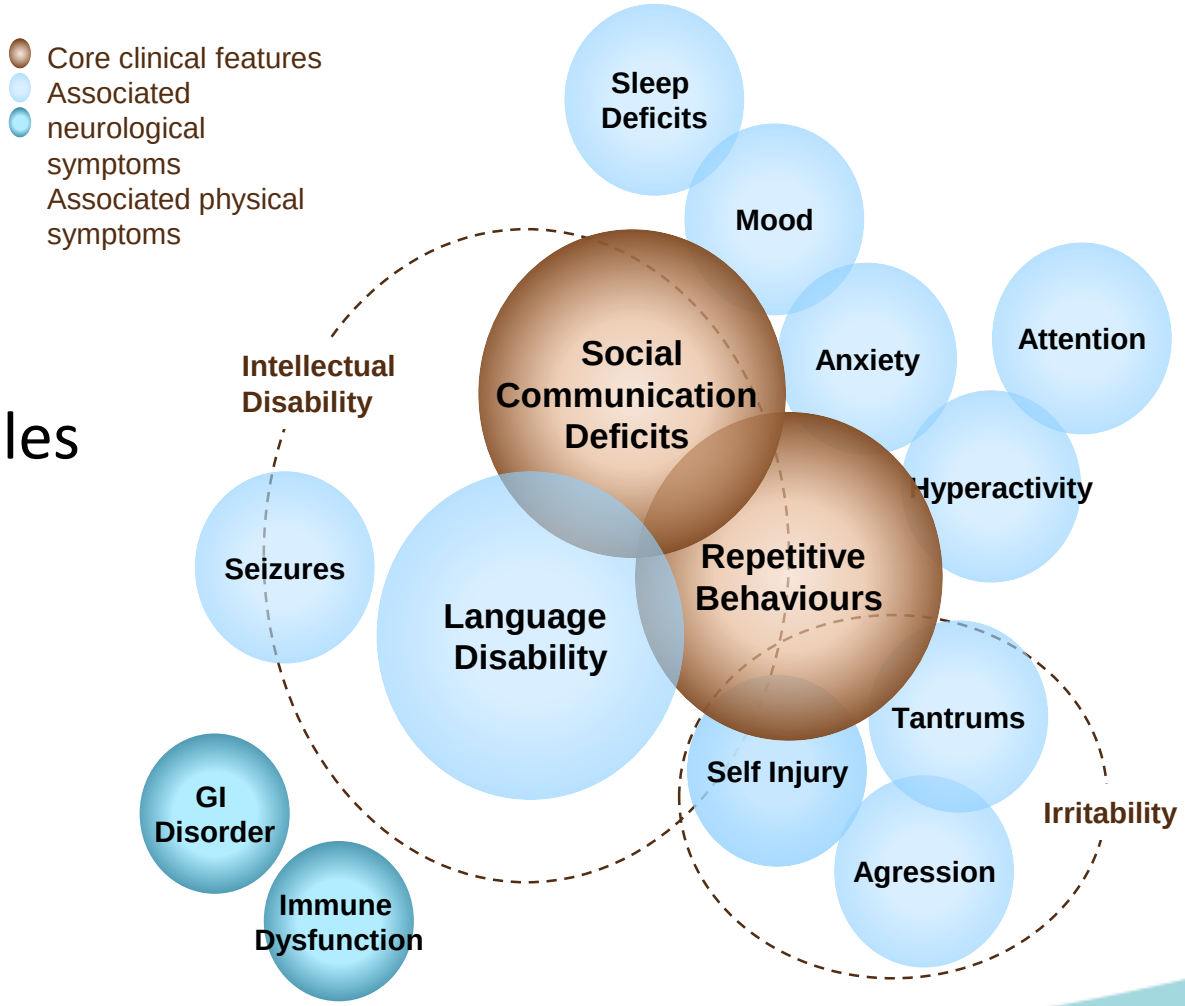
## Gender differences

- 4x more frequent in males versus females

## Strong Genetic link

## Treatment

- No treatment for core symptoms
- Risperdal and Ability for irritability

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**High medical need with no therapy available**

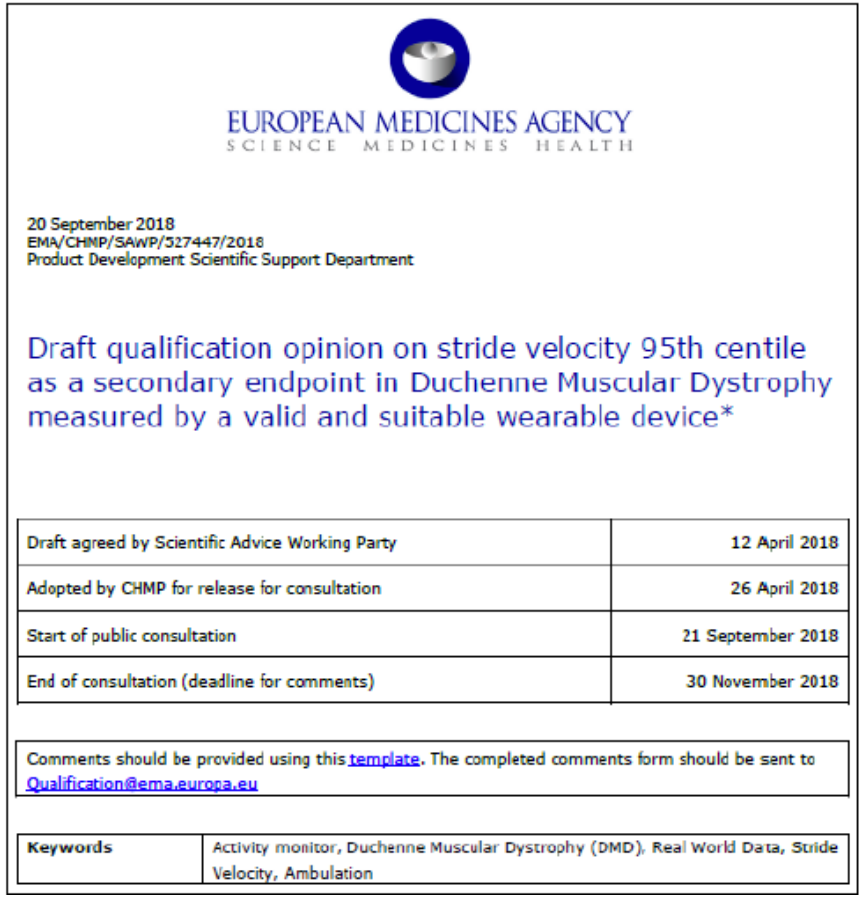
# Autism Spectrum Disorders

*Europe & the World @ 2011*

- No major strategy defined within Europe
- No major or concerted efforts in drug discovery
- No validated clinical endpoints
- No regulatory strategy
- Late diagnosis and poor awareness (adults)
- Poor knowledge of patient needs across life-course
- Wide range in treatment strategy with no evidence of efficacy

# Novel Endpoints in Duchenne's Muscular Dystrophy

- Existing primary regulatory endpoints
  > 100 years old (6 MWT, 4SC)
- Existing enpoints have major weaknesses:
  - Sub optimal sensitivity to functional change
  - Subject to observer and other error, manipulation etc.
- Qualification of 95$^{th}$ percentile stride velocity using wearable device as outcome measurement
- Patient function can be monitored at home
  - high ecological validity
- Draft guidance as a 2° endpoint for regulatory decision making



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*

| | |
|---|---|
| Draft agreed by Scientific Advice Working Party | 12 April 2018 |
| Adopted by CHMP for release for consultation | 26 April 2018 |
| Start of public consultation | 21 September 2018 |
| End of consultation (deadline for comments) | 30 November 2018 |

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

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|---|---|
| **Keywords** | Activity monitor, Duchenne Muscular Dystrophy (DMD), Real World Data, Stride Velocity, Ambulation |

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