Adoption of digital endpoints and telemedicine in clinical trials

Validation and acceptance

Pediatric studies CHDR – Juliana Children's Hospital

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Pediatric clinical trials

MEDICIJNEN MOETEN MEER OP KINDEREN WORDEN GETEST

Esther Janssen / maandag 25 februari 2019, 19:00

Update: maandag 4 maart 2019, 13:08

In Nederland worden zo'n 750 verschillende medicijnen aan kinderen voorgeschreven, maar voor de helft geldt dat er nooit onderzoek is gedaan naar de effecten op kinderen. Er is nu een website die kinderen en hun ouders helpt met de keus om wel of niet mee te doen aan wetenschappelijk onderzoek.

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Barriers in pediatric clinical trials

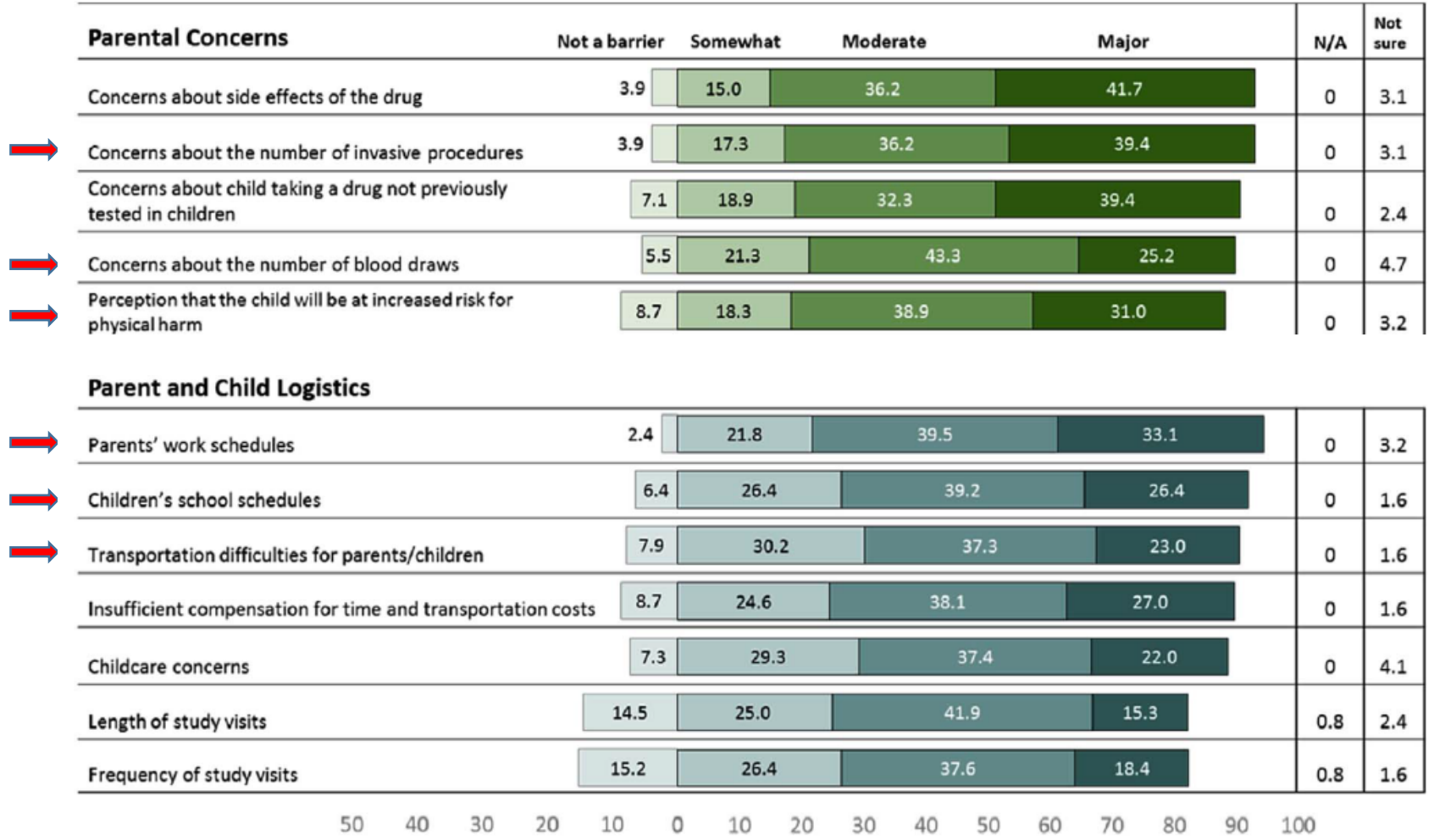

Fig. 2. Provider perceptions of potential parental concerns and parent or child logistical barriers to pediatric clinical trial implementation.

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Contemp Clin Trials Commun. 2018 Mar; 9: 7–12.

Solution?

- Employ non-invasive measurements
- Move trials to the children's home
- Use technology for automated data collection

- **CHDR MORE® - Trial@home platform**

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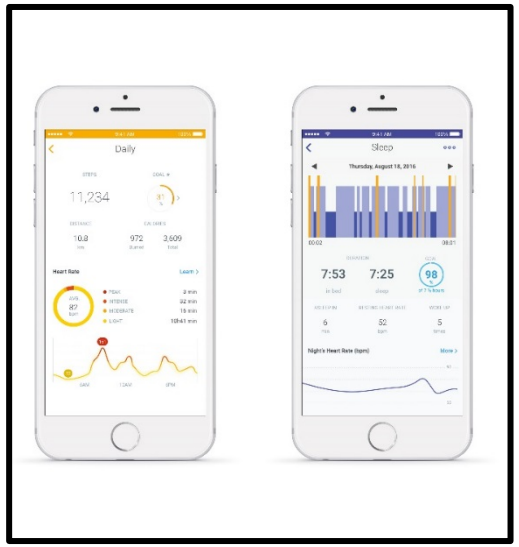
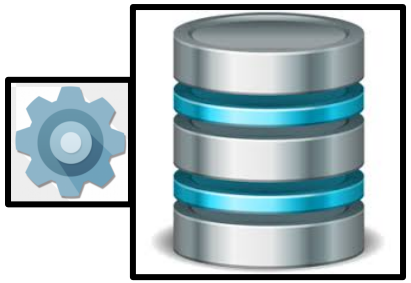
MORE® platform

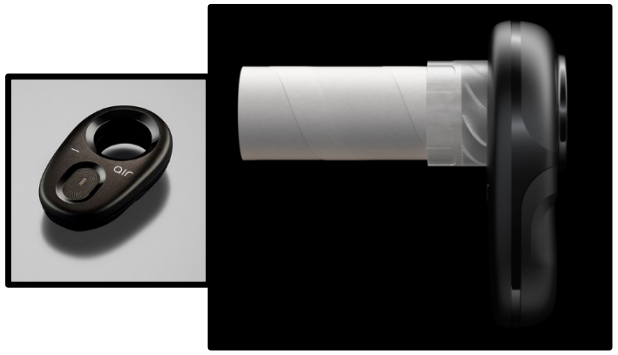
Withings Health platform



Smartphone
MORE® app



Promasys® ePRO



AirNext spirometry

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Validation of new endpoints

- Analytical validation
  - Are the devices reliable?

- Clinical validation
  - Are the devices tolerable for children?
  - Is there a clear difference between healthy and ill children?
  - Can devices detect health events?
  - Is there a correlation with existing measures of disease activity?

- Can devices detect effects of interventions?

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Ongoing studies

- Home-monitoring in chronic pediatric disease
- Home-monitoring in acute lung disease
- Home-monitoring in healthy children
- (Home-monitoring of crying and coughing)

Juliana Kinderziekenhuis

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Logistics

@ home inclusion

Education

Training

Baseline measurements

@ home measurements

21 – 28 days

Daily questionnaire
Watch + disease specific devices

@ home end-of-study visit

Device pick-up

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Preliminary results - Reliability

- Watches break – but less than expected
- Portable spirometry bias (FEV1 / FVC) = 0 L
- Confirmed expected associations
  - Physical activity – Reported activity
  - Physical activity – Heart rate
- Initial ‘Child diseases’ - technological problems

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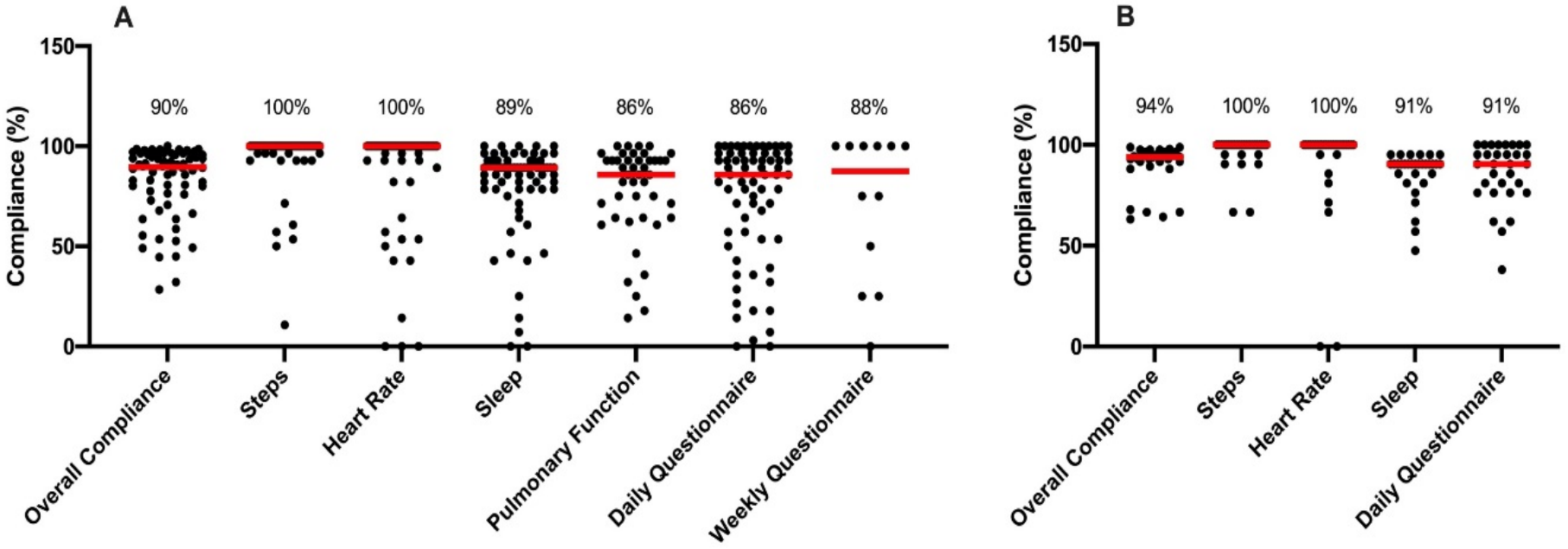
Preliminary results - Tolerability

- 300 children included
- Drop out rate of 4%
- 93% of remaining indicate they would participate again
- Overall median compliance to study tasks ≥ 90%



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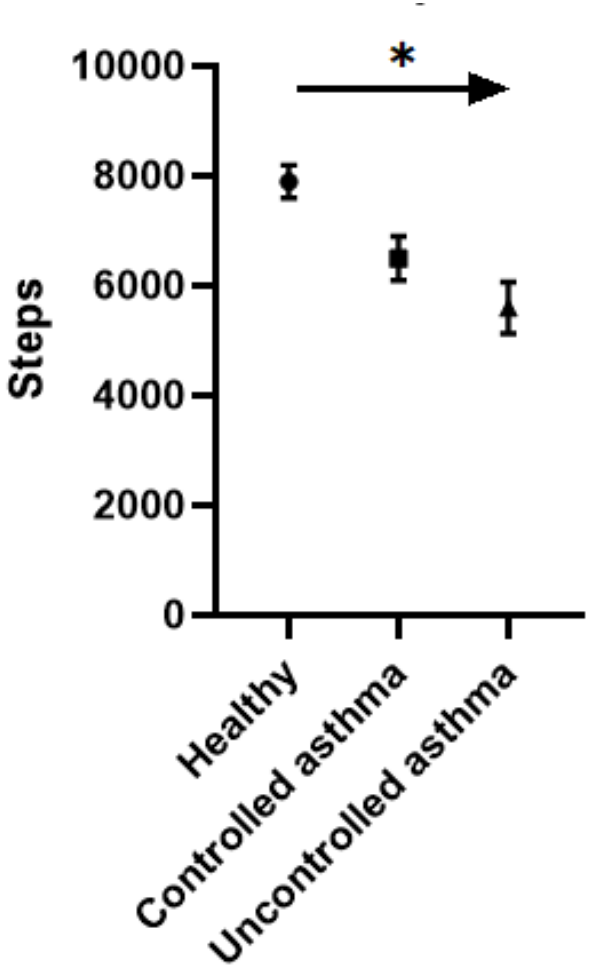
Preliminary results – Difference healthy and ill?



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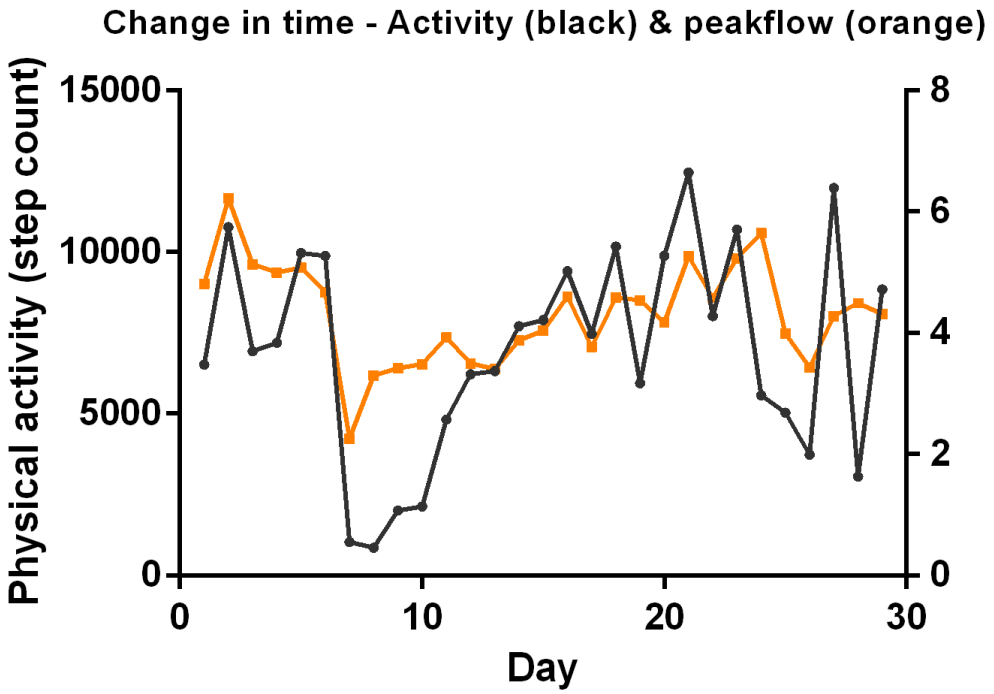
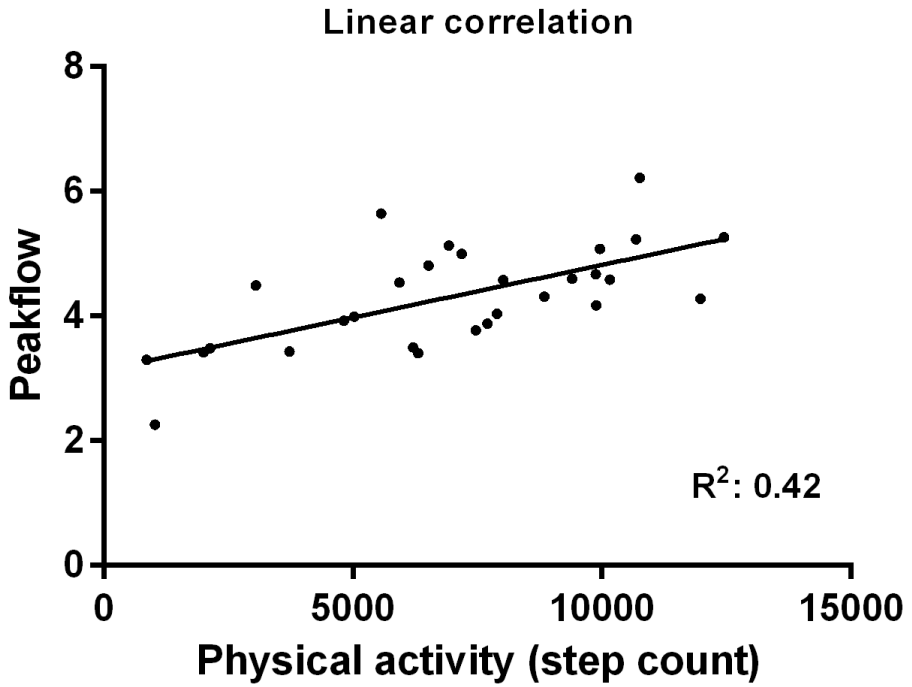
Preliminary results – Detection of health events?





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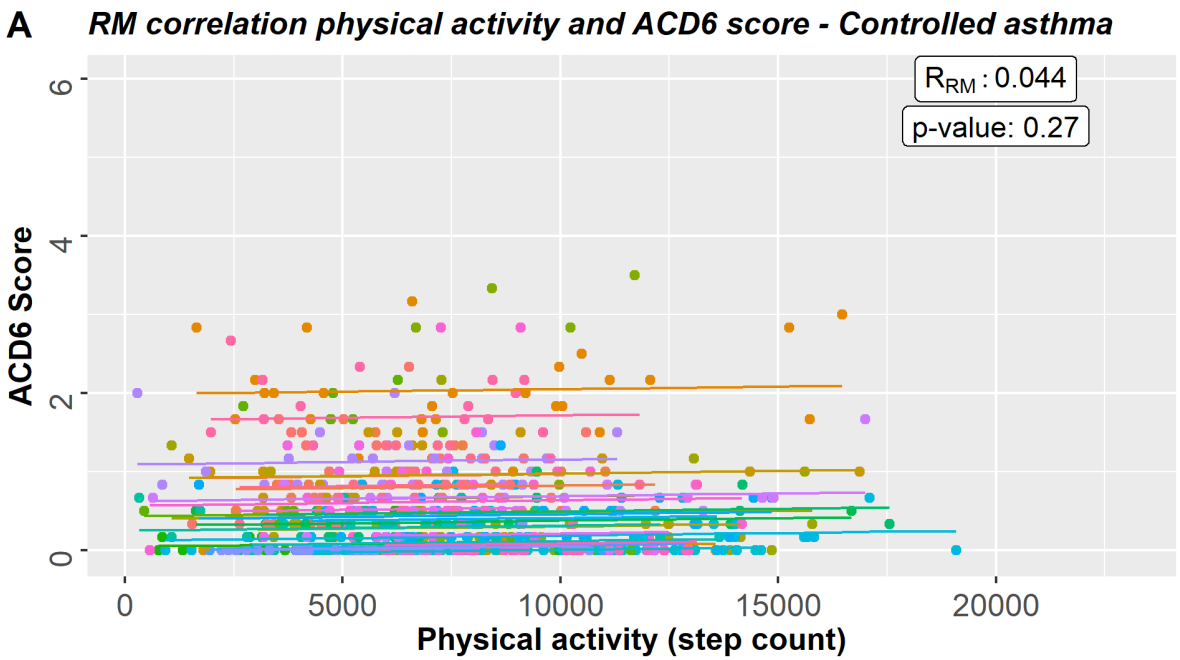
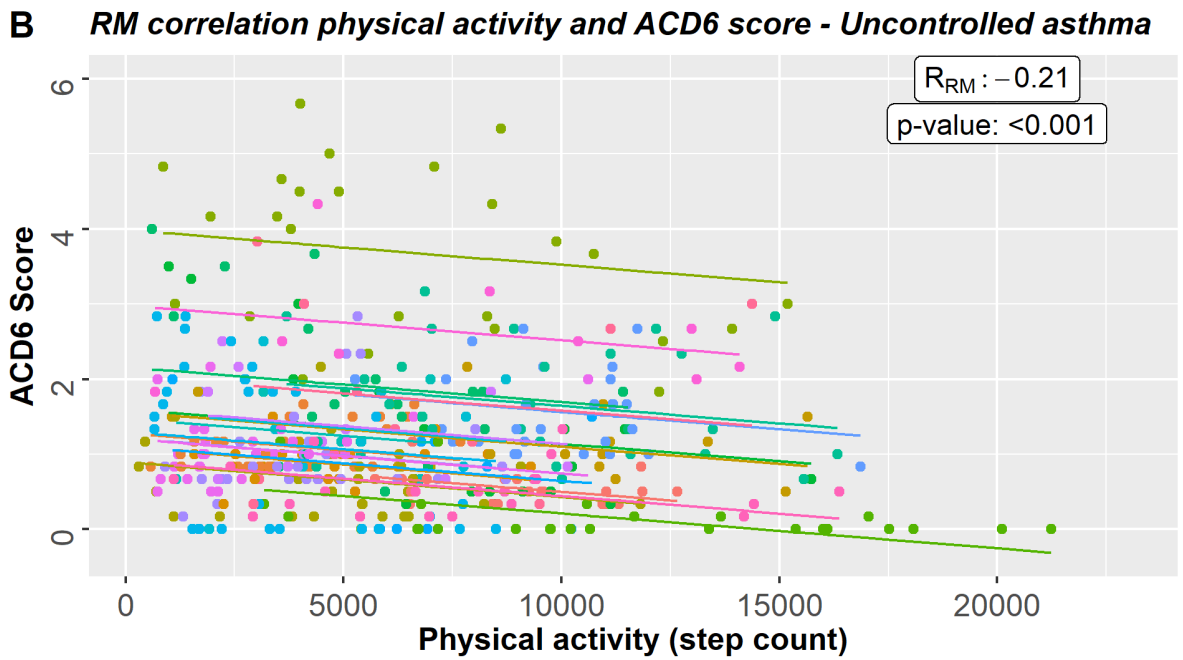
Preliminary results – Correlation existing endpoints?





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Conclusion

- Technology can improve clinical research
- Non-invasive and @home measurements via CHDR MORE®
- Lower barriers for pediatric clinical trial participants
- Preliminary results show promise in pediatrics
- Future:
  - Ongoing studies must be completed
  - Resulting candidate endpoints should be evaluated in interventional clinical trials

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