

# Can rare disease trials be redesigned to be more patient centric using wearables?



Authors: Marie Mc Carthy<sup>1</sup>, Pavel Lebesle<sup>2</sup>, Cynthia Dukes<sup>3</sup>, Valerie Legrand<sup>4</sup>, Willie Muehlhausen<sup>1</sup>,  
 1.ICON PLC, Dublin Ireland  
 2.ICON Clinical Research s.r.o., Prague, Czech Republic  
 3.ICON Plc Sugarland, TX, USA  
 4.ICON Clinical Research sarl, Nanterre, France

## Background

The limited number of Disease Specific Centers of Excellence in any given country, require those patients who wish to participate in clinical trials, to travel long distances with their families to complete clinical assessments.

This additional burden is one of the main barriers for patient recruitment and retention in clinical trials and this in turn impacts the development of new therapies.

An innovative approach to patient centric trial design is needed to address the specific needs of individuals living with rare diseases, many of whom are physically and/or mentally disabled.

Medical devices and sensors are available that could be used in the patients home to enable a patient centric approach by collecting ambulatory biometric assessments remotely.

## Objectives

To identify the feasibility of “place-shifting” rare disease trials from trial sites to patients homes using device, sensors and home care teams

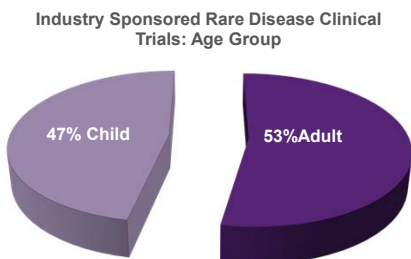
## Methods

Clinicaltrials.gov was mined for rare disease trials and the outcome assessments were identified and categorized into physiological measured that could be captured using readily available sensors and devices.

A market scan was conducted to identify medical grade digital technology that had the ability to capture the required endpoints used in the rare disease outcome assessments.

## Results:

1403 trials identified in Clinical Trials.gov (10/01/2018). This selection was filtered to identify 159 Industry funded biopharmaceutical trials (Drug, Biologicals, Genetic) studies. 47% of these studies focused on children.



## ICON Approach to remote monitoring

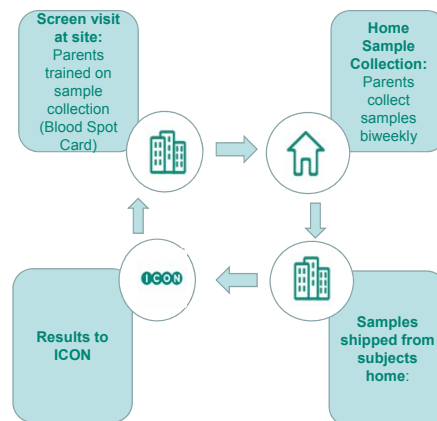
**Sensor Suite Proof of Concept** : Commercially available devices and sensors were deployed to healthy volunteers located in non-clinical settings in 3 different countries. A suite of wearables and sensors can be combined and deployed in a non-clinical home setting, tailored to specific therapeutic areas (Figure 1).



**Figure 1:** A suite of wearables and sensors can be combined and deployed in a non-clinical home setting, tailored to specific therapeutic areas.

## Case Study 1: Rare Metabolic Disease in Young Children. Collection of blood sample at patient’s home:

In this study very young children required blood samples to be taken bi-weekly potentially placing significant burden on the parents. A novel process was implemented where by the parents were trained to take a heel prick blood spot sample which was then collected directly from their homes. This alleviated the travel burden on families and allowed the sample to be collected in a familiar environment for the subject. Samples shipped from subject’s home in a blinded fashion with parents contacting the courier company (Figure 2).



**Figure 2 :** Travel burden on families and patients can be alleviated by allowing the blood sample to be collected at patient’s home.

## Results Continued:

- 49% of the studies had outcome assessments that could be collected in non-clinical setting by a combination of devices and home care teams . Of these 10% had assessments such as vital-signs, lung function, and physical function that could be assessed remotely by wearables and sensors (Table 1).

- 44% of the trials had study endpoints that required site specific outcome assessments e.g. specialist equipment or clinical assessment or examination.
- 7% of trials had no outcome assessment

Assessment	Connected mhealth Devices
Lung Function Tests	• Smart Spirometers
Physical Function and Sleep	• Actigraphy devices, Physical Activity monitors
Vital Signs	• Smart Blood Pressure Monitors • Smart Weighing Scales • Oximetry • Vital Signs Patches (Continuous Heart rate, Respiration Rate, Heart Rate Variability). • Smart Thermometer

**Table 1:** List of Devices that can be used to capture relevant physiological measures in non-clinical settings.

## Conclusion

When reviewing existing trial data from rare diseases studies it has become clear that in order to create more patient centric trials a combination of devices, sensors and ePRO’s and home nursing services is required.

## The following additional insights were observed:

- It is possible to reduce need to travel to sites in 50 % of the combining collecting patient outcome assessment in non clinical setting such as sensor data, ePRO and blood sample collection. (Sensor Suite Proof of Concept ) (Case Study 1).
- Continuous biometric data captured in real life, which be mined for addition clinical responsive signals and facilitate the identification of new digital biomarkers and endpoints that may prove to be more meaningful to those living with rare diseases.

Although further research is required to assess the burden and feasibility of this approach for different populations we believe that when developing a protocol for rare disease patients who have particular difficulties with travel consideration should be given, where appropriate, to the use of digital technologies and remote monitoring .

## References

1. McCarthy M, et al Remote Monitoring of Patients in Clinical Trials. Applied Clinical Trials Sep 12, 2016. <http://www.appliedclinicaltrialsonline.com/remote-monitoring-patients-clinical-trials>