RDR Challenge
Development of a non-invasive tool for measuring rare disease patient mobility in daily living

INDUSTRY SPONSORS

- Chiesi Farmaceutici S.p.A. (Italy)

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- CSL Behring (Australia)

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AIM
To develop a set of coordinated non-invasive tools for measuring rare disease patient general movements distinguishing between voluntary and involuntary movements (e.g. by distributing movement-sensors in patients' home, on their body, on the wheelchair...)

BACKGROUND AND RATIONALE

- Rare disease patients-families-caregivers face important challenges every single day their life. Although enormous progress has been made by increasing international cooperation in the field of clinical and scientific research as well as by sharing of scientific knowledge about rare diseases, there is still a strong need to harvest disease-related information by monitoring patients' behavior and their symptoms**.**
- Mobile health technologies such as wearables, wireless medical sensors, apps are real-time registries that can help in determining rare disease patient best care and guaranteeing a tangible improvement of their quality of life.
  Despite current technological level, there is a lack of integrated systems for collection of mobility information in free daily leaving distinguishing between spontaneous movements and assisted mobility that can generate data suitable for regulatory-accepted patient relevant outcomes
- Benefits for rare diseases
  The availability of a such a tool has the potential to support the improvement of the quality of life of patients care and clinical outcomes by measuring physiological performance (e.g., movement and vital signs) as well as facilitating the assessment of new drugs benefits. In addition, remote assessment of movements offers a tangible advantage as they can reduce travel to study sites for patients and families and increase patient access to research studies. Accurate daily mobility assessment can also help in interpreting overall patient quality of life especially when associated with additional information (e.g. use of pain killers) and can support the estimation of patient independency.

HORIZON SCANNING

- **APARITO** company (https://www.aparito.com/) provides wearable devices and disease-specific mobile apps to provide remote patient monitoring outside of the hospital environment. They are very much experienced with lysosomal storage disorders.
- **MC10** company (https://www.mc10inc.com/our-story) is another provider for wearable Sensors for patients. They have recently collaborated with the University of Rochester (USA) on a study employing Multiple Wearable Sensors in Parkinson and Huntington Disease Individuals: 5 accelerometer-based sensors on chests and limbs were applied for standardized in-clinic assessments and for 2 days at home. The study's aims were to determine the feasibility of using these sensors and the activity (lying, sitting, standing, walking) of participants (Adams et al., Digit Biomark 2017;1:52– 63).

- **ABIOSENSICS** company (http://www.biosensics.com/) develops Wearable sensors for clinical trials (for example: https://clinicaltrials.gov/ct2/show/NCT03599076 for measuring motor impairment in Huntington disease (HD) patients).
- **BENEUFIT** company's technology does not use sensors but just computer vision and machine learning (https://www.beneufit.com/). This is a quite new technology field but we can think if it is worth to shape the challenge in order to be included.
- An interesting project from IMI is the **RADAR-CNS** – IMI project https://www.imi.europa.eu/projects-results/project- factsheets/radar-cns.
  The RADAR-CNS project aims to develop new ways of monitoring major depressive disorder, epilepsy, and multiple sclerosis using wearable devices and smartphone technology. The key goal of the project is to improve patients' symptoms and quality of life and also to change how these and other chronic disorders are treated.

TIMELINES/MILESTONES AND DELIVERABLES

Stage 1 (M18):

- Prototype finalized (6 months for the state of the art and user requirements analyses; 12 months for software programming and fine tuning in parallel with 12 months for preliminary testing and prototype finalization)

Stage 2 (M30):

- Improved and validated set of tools + CE mark obtained

EXPECTED CONTRIBUTION AND EXPERTISE

SME in the field of mobile health technologies is the perfect target of the call, in particular in the selection and/or adaptation of existing technologies in the field of sensors and in the integration of data. People able to generate software for integration of data are also necessary.
Importantly, since patient involvement in the design and set up of these devices represents an added value to the project, the consortium members should be able to offer expertise and support for facilitating patient involvement in the project.

TOTAL BUDGET: 575.000 €

Contribution from the sponsors

In kind

- Chiesi
  Support the involvement of patients in the project
  Help in the definition of technical and regulatory requirements
- CSL Behring
  Expertise in clinical data management
  Expertise in e-clin operations

Financial

Project Name	Total budget (euros)	N° of industrial partners	Min % cash contribution from industrial partner	Cash contribution Industrial partners included in total budget
Mobility project	575.000	2	30%	100.000 (Chiesi) + 100.000 (CSL Behring) (53%)