RDR Challenge
Characterize Rare Bone Disorders (RBD) Mobility Challenges in Real World Setting

INDUSTRY SPONSOR
Ipsen
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AIM
Develop full-body automated mobility assessment tool(s) to assess real-life mobility challenges in people living with RBD, to be compared vs available disease specific patient- and Health Care Professionals (HCP)-reported mobility assessments. Capturing these real-life data could help determine if patient characteristics or environmental conditions could be used to predict mobility outcomes and therefore open possibilities for preventive or corrective interventions, including home and assistive devices design.

BACKGROUND AND RATIONALE
RBD include a variety of disorders of bone formation, modelling, remodeling and removal, and defects of the regulatory pathways of these processes. The severity and progressive nature of some of these disorders such as fibrodysplasia ossificans progressiva (FOP), osteogenesis imperfecta (OI), or achondroplasia lead to mobility limitations. However, little is known about the mobility challenges individuals with these rare diseases are facing, potentially because their condition may limit their involvement in research studies that require frequent visits and/or travel over long distances.
Several tools and scales are now available and used in clinical practice or in clinical trials setting to assess patient- and HCP-reported mobility and joint involvement. Although simple, and rapidly-administered, these tools give only a snapshot of challenges patients are facing and do not reflect a 24/7 measurement in patients' home health care environment.
Digital technologies such as visual sensors and wearable devices have huge potential to measure mobility in the real-world setting. In parallel, the advances in home design and advanced robotics may offer new solutions to support these patients in daily living.

BENEFITS FOR RARE DISEASES
Better understanding of the daily mobility challenges will open possibilities to develop novel endpoints for clinical research to better understand the natural history of RBD and accelerate the development and approval of new therapeutic approaches aiming at preventing mobility decline, or at restoration of function and mobility in these patients. In addition, a short to mid-term benefit could be in designing better home and assistive devices with an immediate impact on patient quality of life.

HORIZON SCANNING
A literature and web search failed to identify similar challenges or available tools. The ones under development are not specific to bone diseases but rather focusing on neurological diseases where the mobility challenges may be different.

- The DIAMOND programme will validate digital mobility assessment, focusing on "real world walking speed" (RWS) as a primary endpoint for a more sensitive, objective measurement in patients' native environment over longer periods of time and with greater granularity than is currently feasible.

- 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)**

Development of a framework to inform larger real-world study of remote mobility monitoring

  - Primary objective: Verification/validation of the tool(s) in real-life setting

- **Stage 2 (M30)**
  - Exploratory objective: Development of adaptable home designs and assistive devices

EXPECTED CONTRIBUTION AND EXPERTISE

- Academic researchers working in the field of RBD
- Patient engagement is key in defining the research priorities and in every step of the project, therefore, involving Patient Organizations and CABs is mandatory
- SME and academic researchers working in the field of mobile health technologies is required
- Involvement of architects and designers (with or without expertise in assistive devices development)

TOTAL BUDGET: 487.500 €
Contribution from the sponsor *(provided all regulatory and legal requirements are met)*

In kind:

- Logistics and organizational support in planning and organizing workshop(s)
- Data sharing (including images, clinical data and Patient- Physician- Reported outcomes);
- Literature analysis (including systematic literature reviews)
- Expert (including Biostatisticians, Digital, Regulatory Affairs) and other technical support (translations, etc.)
- Support presentation of the results at relevant congresses and open access publications in peer reviewed scientific journals.

Financial :

Project Name	**Total budget (euros)**	**N° of industrial partners**	**Min % cash contribution from industrial partner**	**Cash contribution Industrial partner included in total budget**
Rare Bone Diseases Mobility Assessment	**487.500**	**1**	**30%**	**112.500 (30%)**