PaLaDIn, a €21 million programme to develop and implement an innovative global, patient centric, data platform to accelerate the development of effective treatments and care for neuromuscular and other rare diseases, is officially announced today.

PRESS RELEASE

Parent Project Italy, Rome, 31st Jan 2024

The Patient Lifestyle and Disease Data Interactium (PaLaDIn), an ambitious Innovative Health Initiative (IHI) funded initiative (with contribution from The FSHD Society, TREAT-NMD and UK Research and Innovation (UKRI); totalling 21 million Euros), has this month been launched to drive innovative real world data collection from patients with rare neuromuscular diseases (NMDs).

A new state-of-the-art data collection platform is being developed to transform how rare diseases are treated. Known as 'The Interactium', the platform will provide insights to accelerate novel drug development, improve patient reported outcome measures (PROMS) and inform health care decision-making at all levels. The learnings from the project will be shared with those working in other rare disease areas via a series of tools and training materials.

The project is expected to provide a major leap forward in integrating different sources of rare disease patient data, including Patient Reported Outcome Measures, patient preferences and data from registries and wearable devices.

Researchers and medicines developers will have access to the data that clearly elicits patients' preferences and needs, which is particularly important in rare diseases where outcome measures and patient preference data is scarce. This means that treatments and interventions are developed to meet the needs of patients, informing better decision-making in healthcare, medicines development and health technology assessments.

"As a FSHD (facioscapulohumeral muscular dystrophy) patient advocate, I am excited to see this initiative announced, which will help to ensure that the patient experience is incorporated into the development and access of medicines and improving our healthcare.

"There are currently no approved therapies for FSHD, and healthcare providers struggle to meet the needs of patients in their healthcare journey. PaLaDIn represents the hope for me and my family, and everyone affected by this disease, that one day soon, not only will effective therapies become available, but we will be able to access them when they do.

"I look forward to my personal and organizational contributions to the work of PaLaDIn and everything it represents to the FSHD and broader neuromuscular disease communities."

- ***Kees van der Graaf, FSHD patient advocate and board member of FSHD Europe***

The PaLaDIn consortium is a collaboration between academic, SME and patient organisations that includes nine partners from five countries. The four-year project brings together key stakeholders with expertise in NMDs, patient advocacy and data science. It presents a unique opportunity to build capacity for the use of patient data from patients with rare NMDs, to ensure their voices are heard in the planning of care, research and throughout the drug development lifecycle.

The project kicks off with the following goals:

- Develop an innovative omni-data platform 'The Interactium' with capability to integrate data from various diverse sources.
- Improve and increase the PROMs available to the NMD community.
- Maximise the utility, interoperability and reusability of the Interactium data so it can be widely used by researchers and industry, for example in clinical trial planning, drug development and understanding of disease.
- Co-create new digital outcome measures using wearable devices and video that meet patient preferences and needs.
- Develop and publish new datasets for two NMDs (Myotic Dystrophy (DM) and FSHD)
- Improve access to clinical trials by taking tools from the Duchenne muscular dystrophy (DMD) and FSHD communities and implementing them in other diseases.
- Take full advantage of the PaLaDIn impact by scaling up the insights gained from NMDs to benefit the wider rare disease community.

This promising approach to improving healthcare decision-making will take learning from existing programmes and projects in Duchene muscular dystrophy (DMD) and Facioscapulohumeral Muscular Dystrophy (FSHD) and translate this knowledge into other disease areas. Throughout the project, outputs including new datasets, Standard Operating Procedures (SOPS), frameworks and PROMS will be widely disseminated to the rare disease field (and beyond)

"We at TREAT-NMD provide a global network of experts in neuromuscular diseases. The strength and expertise of our network, and all nine global partners working together to deliver the PaLaDIn project, means that we can collaborate to transform the NMD field.

"The mission of this consortium of partners is to create a data platform that will help to pioneer effective treatments, delivering the best care for those living with NMD and other rare diseases across the world.

"One of the biggest challenges we face in our field is the lack of patient data. PaLaDIn is designed to tackle exactly that challenge, working with patients, healthcare professionals and researchers to collate impactful data that can make a big difference in rare disease diagnosis and treatment."

- David Allison, CEO of TREAT-NMD Services Ltd

PaLaDIn will advance knowledge in the rare disease field and address known bottlenecks and hurdles including lack of data from patients living with a rare disease, limited interoperability and reusability of data, low knowledge about diseases, disease heterogeneity, scarcity of PROMS, low numbers of patients.

It is expected that, as a result of the PaLaDIn initiative there will be:

- Up to 4000 patients worldwide who submit data to The Interactium
- New frameworks to test the value of co-creation will be created
- More effective and patient-centred health care solutions and research developed
- Greater de-risking of trials for companies due to the availability of FAIR data
- Development of PROMS, and digital tools to give insights on the burden of disease

These expected outcomes will ultimately contribute to increased patient centricity and improved decision making, outcomes and healthcare solutions.

PaLaDIn has selected four use cases at different stages of development to demonstrate the viability of the newly created platform. Each will use case test the feasibility of using patient data from the Interactium to:

1. Inform regulatory decision making
2. Monitor patient care
3. Create standards of care
4. Facilitate clinical trials

These use cases will have a patient-centric approach and test elements of the platform and the wider project to optimise its tools and services.

The 9 partners are:

Parent Project APS (**Coordinator**)- Rome, IT
TREAT-NMD services Ltd **(Scientific Coordinator) –** Newcastle, UK
Newcastle University- Newcastle, UK
Ludwig Maximillan University of Munich- Munich, DE
Duchenne UK- London, UK
University of Amsterdam Medical Centre- Amsterdam, NL
FSHD Society- Randolph, USA
Aparito BV – Leiden ,NL
Leiden University Medical Centre, Leiden, NL

"Until few years ago, patients with Duchenne and Becker muscular dystrophy and their families did not even know that there was aggregated data or that these could be used.

"Knowing that today, Parent Project APS is leading a trans-European project aimed at collecting data relevant to patients and their families that can reflect their real lives is a dream come true."

- **Ezio Magnano, President of Parent Project aps.**

Links:

Website: https://www.project-paladin.eu/
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Linked in: @eu-paladin

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