

Rare Disease Research Partners

Supporting research and access
to treatment for people living
with rare conditions



SERVING RARE DISEASE COMMUNITIES

Contents

| | |
|---|----|
| Who we are | 3 |
| What we do | 4 |
| Our philosophy | 4 |
| Core Services | 4 |
| Partners in action | 5 |
| Research and evidence | 7 |
| Medical communications | 8 |
| Consultancy | 9 |
| Clinical Trials | 10 |
| Counselling for clinical trial participants | 12 |
| Access to new treatments | 13 |
| Contact | 15 |



“ Everyone affected by or working in the area of rare disease faces a multitude of complex issues. These require clear thinking, broad understanding and a deep knowledge of the science and evidence.

Our team offers all this plus the experience, dedication and passion to make a life-changing difference to people living with rare conditions.

Please contact us to find out more about how we can help you and your work.

”

Bob Stevens
GROUP CHIEF EXECUTIVE OFFICER

Who We Are

We're a team of healthcare experts, researchers, scientists and practitioners supporting patients, families, communities and companies working in the field of rare disease.

Rare Disease Research Partners is a wholly owned, not-for-profit subsidiary of the UK-based **Society for Mucopolysaccharide Diseases** (MPS Society). We reinvest any surplus made from our services to support the Society's mission to transform the lives of patients through specialist knowledge, support, advocacy and research. Since its creation in 1982 the MPS Society has become a leading organisation supporting patients with rare metabolic diseases in the UK.

Mucopolysaccharide (MPS) diseases are a family of around 40 rare, life-limiting disorders that can affect both children and adults. Mucopolysaccharides are long chains of sugar molecules that help to build bones, cartilage, skin, tendons and other essential body parts.

People with MPS diseases do not have enough of the enzyme needed to break down used mucopolysaccharides, which can then accumulate within cells to cause progressive damage.



Globally, the diseases are known to affect approximately one in 25,000 births, although with mild instances often going unrecognised or misdiagnosed, the true incidence is likely to be much higher.

We serve the rare disease community. We do this by applying the knowledge and experience gained in MPS and other lysosomal diseases and delivering a specialised service to a wider community.

Rare Disease Research Partners

Our company was founded in 2012 to boost research and improve access to care and treatment for MPS and wider rare disease communities. Starting with just two staff supporting patients taking part in clinical trials, we've grown and diversified to offer a comprehensive range of services for pharmaceutical and patient organisation clients.

We put patients at the centre of our business to deliver unique research insights, outstanding support for people taking part in clinical trials and access to new treatments. We help patients with reimbursement and provide expert rare disease communications.

Supporting reimbursement

Easing the burden of clinical trials

Patient focus

Unique data



What We Do

We provide research, advice, communications and support for people to access innovative treatments and clinical trials.

Our philosophy

We believe in and stand for:

PATIENTS FIRST

We maintain a unique and specialised understanding of patients and their rare diseases in order to deliver an outstanding service.

COLLABORATION

We build strong relationships with partners working in the rare disease field to achieve the best outcomes for patients, healthcare professionals and the pharmaceutical industry.

EXPERTISE

We combine our professional skills and knowledge with taking the time to understand our clients' individual needs.

INNOVATION

We are proud to be the first service of our kind to have grown from a patient organisation, the MPS Society.

Core services

This brochure gives more detail about our work in:



Research and evidence - on the impact of rare conditions for patients and their families



Medical communications - to raise awareness and understanding of rare diseases, from patient-friendly materials to scientific publications



Supporting clinical trials - improving access to worldwide clinical trials through personalised support for patients and families



Access to new treatments - helping patients to receive ground-breaking therapies by supporting technology appraisals and managed access programmes with bespoke data, analysis of patient outcomes and results for stakeholders



Specialist consultancy - providing specialist knowledge and expert advice on all aspects of rare diseases.

Partners in action

This case study shows how our research and advocacy raised awareness of an ultra-rare disease to improve information and care for people affected by it.

Less than one in a million

The disease

Mucopolysaccharidosis type VII (Sly disease, MPS VII) is an extremely uncommon, multi-symptom disease, which presents particular challenges with diagnosis, management and care. People with the condition can have a wide variety of serious and chronic problems with physical and mental development, sensory impairment and reduced life expectancy.

Worldwide, it is estimated that MPS VII has a frequency of less than one in a million births. There are only 30 known cases in Europe, and the disease is the UK's rarest mucopolysaccharidosis, with an average of only one affected birth every ten years.

Diagnosis may take several years, with early symptoms often being non-specific or mistaken for other childhood conditions. This can delay or prohibit patients' access to clinical trials and treatments to improve their health and quality of life. It also means that there's a lack of reliable information about the impact and burden of the disease on patients and caregivers.





The project

Rare Disease Research Partners collaborated with patient advocacy groups across Europe to design and undertake the first survey exploring MPS VII from the perspectives of patient and caregivers. The project connected with a sample of people in Germany, Spain, The Netherlands and Turkey to consider their experiences of:

- Early and current symptoms
- Pathway to diagnosis
- Burden of illness
- Impact on caregivers' lives and opportunities
- Clinical contact and treatment
- Availability and use of health, social and educational support.

Findings and action

The survey identified a need for greater awareness of the early signs of MPS VII, with improved information for parents and caregivers during and after diagnosis. We found that the disease presents a significant burden to patients, caregivers and providers of health, social and educational services. Responses highlighted variable levels of access to relevant information and support across Europe, and limited availability of genetic counselling in some countries.

Based on these findings, we raised awareness through a targeted publication and poster presentations at international metabolic disease and paediatric conferences.

Better information means faster diagnoses and improved care for people living with rare diseases.

Research & Evidence

Effective research into rare diseases relies on a solid understanding of the unique challenges faced by patients and their caregivers. We specialise in studying the needs of these small populations and promoting their interests.

Our approach

We offer:



Insight

Direct from patients, as the cornerstone of novel therapies for rare diseases.



A tailored approach

Using patient surveys, interviews, focus groups and other flexible methods to support studies, from initial concept to reporting, publication and promotion.



Expertise

Customised research into:

- The impact of rare diseases
- Diagnostic journeys
- The burden on caregivers
- Patient-reported outcomes
- People's experience of treatment.



Links with patient communities

Close partnerships with rare disease patient organisations worldwide, which allows us to work across borders while retaining our focus on rare and complex diseases.



“

It's fantastic to take a research project all the way from design to final publication. We tailor our communication plans to the needs of the audience, from preparing posters and showcasing our clients' and own research, to developing disease awareness programmes.

”

Dr Eva Raebel
SENIOR MEDICAL COMMUNICATIONS
AND RESEARCH ASSOCIATE



“We’re always excited when a new project comes our way. We like nothing better than planning research, exploring the evidence and communicating results with our pharmaceutical clients, patient organisations and clinical colleagues to make a real difference to patients’ lives.”

Alexandra Morrison
HEAD OF RESEARCH AND MEDICAL
COMMUNICATIONS

Medical Communications

Our experienced scientific and industry-expert team delivers high-impact content across a range of channels, from patient-friendly material to scientific publications.

Our voice

We deliver:



Patient materials

Making sure that the content, language and chosen channels are suitable for patients and patient organisations.



Research publications

Planning, content and submission to peer-reviewed journals and international conferences.



Symposia and meetings

Showcasing our clients’ work and own research, including posters and multimedia presentations.



Educational resources

Awareness programmes based on our in-depth knowledge of rare diseases, plus strategic advice to help clients reach their target audiences and stakeholders.

Consultancy

We can advise and assist on engaging effectively with public health bodies, establishing advisory boards, collecting patient-reported outcomes or involving patients in research and studies.



Engaging patients

Guidance on how to understand and overcome the barriers to engagement associated with rare diseases (for example, how a limited population can affect research, avoiding survey fatigue and maximising patient retention in clinical trials).



NICE evaluation

Strategic and practical support through the NICE evaluation process for rare disease treatments, including health economic models, submission dossiers and approaches to increase the chance of a successful outcome.



Advisory boards and focus groups

Relationships with rare condition opinion leaders and international patient groups, plus advice on how to plan and deliver advisory boards or focus groups on any topic.



Stakeholder contact and communications

Help to engage with rare disease individuals, groups and communities to gather data and insight. Support for connecting and communicating with NICE, NHS England and other public health bodies.



“

We've supported managed access agreements since they were first introduced for rare diseases as part of the Highly Specialised Technologies evaluation process. We pride ourselves on delivering comprehensive and robust data, while making it easy for patients to participate.

”

Samantha Wiseman
MANAGED ACCESS AGREEMENT
COORDINATOR

Clinical Trials

From initial screening and throughout the duration of a clinical trial up to the final safety visit, we're always on hand to offer personal support to participants and their families.

Our support

We provide:



Sponsor support

Advice and support to trial sponsors, including travel and reimbursement guidance, key issues reporting and independent medical opinion.



Dedicated coordinators

Continuity of contact and support from a dedicated Rare Disease Research Partners coordinator.



Financial security

Prompt settlement of study-related expenses to reduce the financial burden on patients and their families.



24/7 assistance

Round-the-clock advice and support to help patients and their families attend study visits, with an out-of-hours emergency phone service.



Personalised travel and accommodation

Liaison with study sites across the world to provide personalised travel and accommodation options for patients with unique and complex needs.



Multilingual support

Access to translators and interpreters in more than 240 languages.



Logistics and relocation

Local and international help for patients with complex needs to join and continue with clinical trials, including support for relocation anywhere in the world.



Increasing retention in clinical trials

With only limited numbers of patients available to take part in clinical trials for rare disease treatments it's crucial to help those who take part stay involved.

Studies show that almost one in five participants of randomised trials withdraw for some reason. The most common factors that lead people to stop being involved are:

- Personal or family circumstances
- Financial constraints
- Conditions not improving
- Location of trials
- Side effects of treatments

When surveyed about their experiences of clinical trials, 38 per cent of patients who withdrew said that attending site visits was stressful, compared to just 16 per cent of those who completed their trial.

An audit of five clinical trials for which we offered specialist support, involving 166 patients across 51 sites, showed that the withdrawal rate was lower than that reported in the literature.

Making it easier to take part in clinical trials and improving patients' experience helps to improve retention rates and strengthens the outcomes from rare disease studies.



“Taking part in a clinical trial can be stressful for patients and their families, but travelling to the clinical trial site and managing expenses does not have to be. We take care of their travel needs and reimbursement.”

Benedicta Marshall-Andrew
HEAD OF CLINICAL TRIAL
SUPPORT TEAM

Counselling for Clinical Trial Participants

Counselling can help to alleviate stress for patients and their family members by working through difficult or problematic life events, addressing distressing thoughts and feelings, or hopes and anxieties about their trial participation.

Our service offers study sponsors the opportunity to provide trial participants with*:

- ✓ Accredited counsellors with expertise in rare conditions
- ✓ An understanding of the demands of clinical trials
- ✓ Valuable additional emotional support
- ✓ Counselling sessions on a case-by-case basis according to the individual's needs
- ✓ A lead counsellor as the primary point of contact for the participant

*Available for English speaking participants in UK & Europe. Some regional exemptions may apply



In partnership with
rareminds
Mental Health for the Rare Disease Community

Access To New Treatments

We work closely with assessment bodies, payors and clinical centres to help patients, patient organisations and pharmaceutical clients to meet the requirements for new treatment reimbursement, including through managed access agreements and technology appraisals.

Managed access agreements

We supply:



Patient-reported outcomes

Expert advice on planning and collecting patient-reported outcomes and quality-of-life measures.



Data collection

On all aspects of patient and treatment information, from individual consent to surveys, interviews and focus groups.



Data interpretation and analysis

Advice on analysing and interpreting results, data and patient-reported outcome measures.



Reports and publications

Detailed reports to NICE, NHS England and other stakeholders, with support to publish and disseminate findings.



Patient support

A flexible appointment system for patients to complete assessments, with interpreters and translation if needed.



Plain language

Materials that explain the complex requirements of managed access agreements in a way everyone can understand and follow, including patient and caregiver booklets.



Multilingual support

Access to translators and interpreters in more than 240 languages.

Technology appraisals

We supply:



Patient organisation submissions

Bespoke research on patients' experience of treatment and the burden of illness on patients and their families to support patient organisation submissions and highly specialised technology appraisals.



Contact

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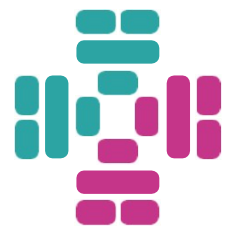
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Please get in touch if we can help you or your organisation to advance research, treatment and support for people living with rare conditions.

Rare Disease Research Partners is the trading name of MPS Commercial, a private limited company, registered number 08621283. We are a wholly owned, not-for-profit subsidiary of the Society for Mucopolysaccharide Diseases (the MPS Society), registered charity in England and Wales number 1143472.

We reinvest any surplus from our services to support the MPS Society's mission to transform the lives of patients through specialist knowledge, support, advocacy and research.

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Rare Disease
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WE'VE SERVED RARE DISEASE COMMUNITIES SINCE 2012