

Research Strategy 2021-2026

The Myrovlytis Trust aims to transform the outlook for rare conditions and ensure that patients affected by rare conditions gain access to the same state-of-the-art technologies, breakthroughs and therapies as those with more common disease.

One of the ways the Myrovlytis Trust can accomplish this is by funding research directed towards new therapies for rare genetic disorders and rare cancers.

This strategy sets out how we aim to achieve this.

Current Challenges in Rare Disease and Rare Cancer Research

More than 7000 rare diseases have been described, affecting 300 million people globally. It takes on average over 4 years for an individual to receive a diagnosis, and even then, only 400 of these diseases have an approved treatment.

Rarity

Due to the low number of people diagnosed with a specific rare condition, many clinicians often see few patients, or infrequently see them. Not only does this mean awareness of the disease is generally low, but it is difficult to build a complete clinical picture, such as prevalence of the disease or an exhaustive list of symptoms. Funders may also be unwilling to support research into a condition that affects such a small number of people. We believe it is important that advances in technologies and tools in the clinic are applicable to other related rare diseases and rare cancers or at least can be used to inform the development of treatment strategies for other related conditions.

Complexity

The combination of rarity and complexity within rare conditions poses additional challenges. Small study sizes can be problematic for designing studies or clinical trials that will produce statistically significant results. It is possible that for enough patients to be recruited to a particular study or trial research may have need to be conducted across multiple countries, with the associated increase in costs.

The Role of the Myrovlytis Trust

By bringing together researchers, clinicians and patients, the Myrovlytis Trust aims to enable co-operation within the community to drive forward research and improve the quality of life of people affected by rare conditions.

The Myrovlytis Trust intends to strategically fund research into our priority areas of BHD, related kidney disorders and osteosarcoma.

Research Principles

The following principles guide the Myrovlytis Trust's research strategy:

1. Relevance to the treatment or cure of rare diseases.
2. Potential to improve the prognosis and quality of life of people affected by rare diseases.
3. Evidence that funding cannot be suitably obtained elsewhere.
4. Originality and excellence of science.
5. Dissemination of research to the scientific community through publication of results, and to the wider population through public engagement in line with the aims of the Myrovlytis Trust to bring

clinicians, researchers and patients together to advance the field.

6. Potential of new advances in technologies and tools in the clinic to be applicable to other related rare diseases.

Research Priorities

Our current priorities focus on two main avenues of research – BHD (and related kidney disorders) and osteosarcoma.

We are particularly interested in funding the following:

- Research to develop new routes to treatment including immunotherapy and gene therapy
- Clinical trials
- Multi-disciplinary projects

Specific to BHD

- Research to investigate the biochemical processes in which folliculin is involved and the function of folliculin

Specific to Osteosarcoma

- Research to develop predictive models of patients' response to therapy and the discovery of new biomarkers.

How will Research be Funded?

The Myrovlytis Trust welcomes applications from researchers worldwide and offers the following types of grant:

- Small/Pilot Grants (up to £20,000 for 1 year)
- PhD Studentships (3.5 years funding for a PhD student, including stipend, university fees and consumables – normally capped at £95,000)
- Project Grants (up to £300,000 for 3 years)
- Public Engagement Grants (up to £5000)
- Travel Grants (up to £1000)

The Myrovlytis Trust believes in empowering patients by making research accessible to everyone. A plain English summary of the research proposal must be provided at application stage, as well as results of the research in due course. This summary will be evaluated by members of our Patient Advisory Board during the review process. In addition, grant holders must provide regular scientific progress reports to the Myrovlytis Trust.

Strategy Review

Annual reports on funding allocation and overall expenditure will be published on the Myrovlytis Trust website. The research strategy will be reviewed every 5 years by the Myrovlytis Trust and the Scientific Advisory Board.