

Scientific Strategy

for the

M Y R O V L Y T I S T R U S T

London, 14th May 2008
JCS

1. Why is the MT funding research?

- 1.1. Ideally, to cure BHD syndrome. Shorter term, to generate treatments for BHD syndrome.
- 1.2. To promote a new model for scientific research. One that maximises the impact of research by encouraging collaborations and the timely dissemination of scientific results.

2. Criteria, one or more of which should be met by research to be funded:

- 2.1. Improve understanding of the molecular and cell biology of folliculin and BHD syndrome.
- 2.2. Maximise the benefit of current research:
 - 2.2.1. Encouraging collaborations
 - 2.2.2. Providing lab resources (see 3.3)
- 2.3. Work towards clinical trials and new drug development.
- 2.4. Bring us closer to an improved therapy

3. How do we fund research?

- 3.1. Smaller project grants.
- 3.2. Larger programme grants

A typical project grant will address a narrower biological question than a programme grant, often over a shorter time period. The amount of a project grant is typically smaller than a programme grant. A project grant would often have just one person working on it: studentship would be a typical project grant, while a grant funding more than one post-doc over several years would be a typical programme grant.

- 3.3. Creating lab resources freely available to all researchers (e.g. cell lines, constructs, mouse lines).

All applications under consideration for funding are sent for peer review. Applications with matching funds available are encouraged.

4. Who do we fund?

- 4.1. Researchers carrying out world class research into BHD syndrome. Research is not restricted to big, established laboratories; high quality applications from small and new laboratories are welcomed.
- 4.2. Funding, for the moment, is restricted research into BHD syndrome. The MT has an aspiration, at some point in the future, to fund research into other rare genetic disorders.

5. Where do we fund research?

- 5.1. Globally. No restrictions.

6. When did we begin and when do we review the strategy?

- 6.1. Started in late 2007. Strategy to be updated annually and reviewed every six months.
- 6.2. There is a Scientific Advisory Board (SAB), which advises the Myrovlytis Trust regarding its scientific direction and focus. The SAB is not privy to all applications – confidentiality, where any actual or perceived conflict of interest arises, is strictly maintained.