

SAB First Meeting Report

for the

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

Conference call

8th May 2008

On the call: Laura Schmidt; Eamonn Maher; John Solly

Taking minutes: JCS

Summary of Discussions:

Where are the gaps in our scientific knowledge of BHD syndrome?

BHD research is in an early stage of development. The links to the mTOR pathway are interesting and there is increasing interest in BHD from established mTOR researchers. However it seems likely that BHD will have multiple functions and so it is important that basic research is encouraged to provide a more comprehensive knowledge of folliculin function and so provide the best opportunities for developing targeted therapeutic interventions. A wide range of research approaches will be required including investigations in model organisms.

How do we develop therapies?

Improved knowledge about folliculin biology will provide the best chance of developing therapies. Although much work needs to be done, the most likely option for novel therapeutic interventions in next 5 years is likely to be with agents that influence mTOR signalling.

Where the MT is today, supporting scientific research:

Details of current MT-sponsored research are available on the MT website. Given that BHD research is at a relatively early stage, MT might wish to emphasise supporting several centres (rather than a single Institution) in order to encourage a variety of approaches as the best way to advance knowledge of BHD biology. In addition, funding of key resources that would be made available at an early stage to multiple laboratories could facilitate collaboration and expedite research. Knowledge that MT will be a long-term supporter of BHD research is also likely to encourage researchers to become involved in the field.