



Research Pathway – Idea to Output

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Research Pathway

- **The Idea**

- **What are some unanswered questions in prion science?**
 - How do they travel from cell to cell, how might that be stopped
- **Do I have the expertise and capacity to answer such a question**
- **Can I try a new approach to answering the question?**
- **Drawing from another field**

- **Would my idea make any difference?**

Research Pathway

- **Has someone thought of it before?**
 - **Read thoroughly, discuss with others**
 - **What can learn from previous research to make for a approach?**
 - **Do I have access to new tools – new animal or cellular models, new technologies like microscopy and imaging, new ways to modify genes**

Research Pathway

- **Can I test the idea ?**

- **Can I design experiments or collect data that would let me know if my idea might be correct?**
- **Do I have the have the appropriate facilities and staff?**
- **Can I bring in an idea from another field?**
 - **Prion amplification technologies for testing for the presence of prions at very low levels**
- **Can I form a collaboration to develop a reliable way to answer a question? If I can modify genes can I team up with someone who is expert in imaging technologies in cells or animals?**

Research Pathway

- How do I check if my idea is correct?
 - **Form a hypothesis**
 - **If my idea is correct, then I predict that X will happen if I do Y to a system (in a test tube, a cell, an organ, an animal)**
 - **I design various ways to test my hypothesis – the experiments**
 - **If X happens after I do various Y actions, then I and others will begin to accept that my idea might be correct**
 - **If I or anyone else does Y and X does not happen the idea or part of it has to change**

Research Pathway

- **How do I do the experiments?**
 - I need to develop a feasible plan of different ways to test the idea (the hypothesis). I might need to test the hypothesis in different cells and difference animals. Not all cells and animals will behave in the same way, so one needs to work up to humans.
 - I have to find a way to get sufficient financial backing to carry out the research plan
 - I need to apply to agencies for research funding

Research Pathway

- **Testing the idea**
 - **Controls and reproducibility**
 - **Essentials of good research**
 - **Prepare alternative pathways and questions in case inconsistent answers are obtained from different methods**
 - **Prepare a budget**

Research Pathway

- **Financing**

- **Large agency – National Institutes of Health**
- **Medium-sized agency – Michael J Fox, Alzheimer’s Society**
- **Small agency – Usually targeted – CJD Foundation**

Research Pathway

- **Why are CJD Foundation Family Grants important?**
 - **Area of research highly relevant to donor interests**
 - **Allow a new person, especially a young person, to enter the field**
 - **Allow a researcher to test an idea that would not be funded in larger agencies because the competition is so fierce**
 - **Rigor of the decision process is very high. Creditability to the research and the researcher**
 - **Allow researcher to get those early results that make pursuing the idea more likely to succeed with larger agencies (getting preliminary data)**

Research Pathway

- **Peer Review**

- **The mechanism that the research system uses to carry out due diligence on research proposals, and publications and personnel awards**
- **Sometimes agencies consider relevance as part of the review process directly**
- **Sometimes agencies consider the views of patients or advocates during peer review process – more often not**
- **Most agencies will accept the recommendations of peer review groups and follow them until they have consumed the available funding**

Research Pathway

- **You receive the funding!**
- **Then what?**
 - **Then you have to do all the stuff that you said you would do in the plan (grant application)**
 - **The pathway is seldom straight. Many of the sub-hypotheses turn out to be incorrect.**
 - **A revised sub-hypotheses or even new overall hypothesis might be needed, but the data gathered will always be useful on the twisting road that is research**
 - **Your data adds to the body of knowledge that helps you and others understand better**

Research Pathway

- **Telling others about the findings and determining if the findings can be applied beyond the research effort**
 - Write a record of your findings (prepare a scientific paper)
 - Where to present findings – What journal? What conference?
 - Maybe file a patent application
 - **Be subject to peer review**

Research Pathway

- The answer to the research question is positive. Then what is next?
- How does the research get moved into practise?
 - Various forms of trials in animals and humans
 - A diagnostic – 7-12 years; \$10-50M
 - A therapy – 7 -15 years: \$10Ms ++++
 - Different costs, different risks, different routes for approval
 - Therapies and rare diseases. Therapies and compassionate use. Can sometimes shorten time frames, but difficult to get large numbers for trials

Research Pathway

- **Repeat the Cycle of planning, applying and so on**
- **If other researchers think you have a good idea many others will follow it in their own experiments – no longer a “small” idea**
- **Likely you will have to adapt or change your hypothesis before it becomes accepted. After enough other scientists cannot prove that your idea is wrong it will become accepted**