



The research charity perspective; hopes, hypes and practicalities

Dr Sandhya Duggal
University of Birmingham

Background

- Why research charities?
- Intermediary position between scientists and patients
- Pathways to clinic
- Little inquiry into their expectations

Background

- Areas of RM activity



British Heart Foundation – RM and cardiovascular research. Funding around £38m in the UK. A new research programme - Mending Broken Hearts



Alzheimer's Research UK – iPSC derived human neurons as models and for in vitro models of pathology. Three drug discovery institutes at Oxford, Cambridge and UCL.



Fight 4 Sight – investment into Retinitis pigmentosa and Stargardt Disease, 'Fulbright Fight for Sight Research Award'

Methodology

- Interviews with 9 medical research charities:

Neurological	Autoimmune	Dermatology	Cardiovascular	Ophthalmology
Parkinson's UK	JDRF	DEBRA	British Heart Foundation	Fight 4 Sight
Scottish Huntington's Association				
MS Society				
Alzheimer's UK				
Motor Neurone Disease Association				

Findings

- Managing media expectations
- Disease specific appropriateness of RM
- Future success of conditions

Managing media expectations

- Media rhetoric of promise
- Narratives of hype characterised by timespan
- Reframing public expectations

“I think it’s important that those results see the light of day, but it’s just the way it sometimes gets presented, I think, that it’s good if organisations such as ours can help try and, you know, temper some of that”

Disease-specific appropriateness of RM

- RM and varied ‘compatibility’ to disease areas

*“ (T1D) - it’s a **perfect disease** for RM because you absolutely know your cells and you know that if you put them in they’re going to have an effect. It’s a bit like Parkinson’s - it’s known that if you were able to replace those cells you would actually be able to have an effect on the disease. So there are some diseases that it’s **really appropriate** for”*

*“So rewiring the nervous system is, frankly, **science fiction** when it comes to conditions like Motor Neurone Disease. It might have more relevance in focal degenerative conditions, let’s say, like stroke or maybe Parkinson’s Disease”*

Expectations of future success conditions

- Practical and theoretical developments needed for RM success
- ‘De-risking’ for future investment

*“So if we can change that balance, **make it less risky**, hopefully, that would bring new investment and new trials and more drive to find RM therapies for Parkinson’s. At the moment it’s quite a **difficult environment** for people who are looking for those new drugs and encouraging pharmaceutical companies to really try new, innovative treatments”*

Conclusion

- Balancing act - hope and hype
- RM for diseases or diseases for RM?
- De-risking RM
- Pathways to clinic



Thank you

- Questions