BioMod Interview schedule template: gene editing and gene therapy [Companies template]

Explanatory Note: main questions are indicated by a single, solid bullet point ‘•’. Prompts, which are only used when needed to stimulate further discussion or if the interviewee has not already discussed a particular topic of relevance, are indicated with a single clear bullet point ‘○’.

1) **Preliminary discussion**

* Brief introduction to aims and objectives of ESRC Biomodifying Technologies project
* Interviewee rights: confidentiality, anonymity and right to withdraw
* Data management plan – interview transcripts with identifying data removed will be deposited in UK Data Archive at end of project
* Opportunity to ask questions about project

2) **Interviewee and team overview**

* Can you tell me a bit about your professional background?
* Roughly how long has your company been working on gene therapy technologies?
* Roughly how many people are employed by the company?
* What do they do [i.e. what are their roles]?
* What sorts of backgrounds and training or skill sets do they have?
  + What are the main scientific / engineering disciplines required to develop gene therapy and its applications?

3) **Current scientific work**

* Can you say a bit about your therapy/product/technique? How novel is it compared to existing therapies/tools/products?
* What are the key technologies and biomaterials that you use?
* What are the main reasons /advantages for using this approach?
  + Time?
  + Expense?
  + Available skill set of employees?
  + Material properties?
  + Ethics?
* How do you procure these materials? (NB we do **not** need names of specific suppliers, but are interested to know if materials are sourced from other companies, hospitals, biobanks etc).
* Are you aware of similar products/therapies/techniques being developed elsewhere?
* Has the advent of CRISPR/cas9 and other ‘programmable nucleases’ had an impact on your area of work?
* Does your company use programmable nucleases in its own work?
  + As research tools?
  + As part of a manufacturing process?
  + As a potential therapeutic option?
  + Or if not, are there reasons why you do not use these tools?
* What stage of development do you regard your work to be at (and in relation to the field)?
* What aspects of your (work/project etc) are established, what is ‘experimental’?
* What are the main ‘risks’ related to your work/projects etc. from your point of view?
* If your work has potential clinical application, how do you envisage this being applied as a therapy?
  + What are the obstacles to translation?
  + Have you started clinical trials?
  + Are there specific problems of doing clinical trials of gene therapy?
  + Are there specific manufacturing / logistical challenges with doing gene therapy? (e.g. access to cGMP facilities)?
  + What are the major markets for your products?
* Do you see gene therapy as something that could one day become a routine clinical procedure?
  + Who would deliver it? Would they need special training? In what?
  + Would hospitals/clinics need special facilities?
  + Would it be able available across the NHS, or only in specialist centres?
* Are suitable animal models available for preclinical testing of your product(s)
* Does your work involve any applications with induced pluripotent stem cells or 3D bioprinting? (our other project case studies)

4) **Network and resources**

* What, if any, types of groups or organisations do you collaborate with?
  + E.g. academics, Contract Research Organisations, hospitals
* Do you provide services to any groups within your organisation or outside it?
* Do you outsource any tasks to other groups?
  + If so could you explain which tasks and why you outsource them?
* What types of contractual arrangements do you need to enter into to pursue your aims?
* Do you draw on any external resources to conduct your research e.g.
  + Bioinformatics?
  + Sequencing/Genotyping?
  + Cell lines?
  + Biobanks?
  + NHS patients /Patient organisations?
  + Manufacturing / Contract research organisations?
  + Support for moving to clinical trials/commercialisation? [e.g. the cell and gene therapy catapult]
* If so, where are these generally based –UK, EU, USA, elsewhere?

5) **Regulation and translation**

* How does regulation impact your work?
  + Probe - Respective influence of MHRA/FDA/EMA, others?
* Do you find the regulatory climate difficult to navigate? How does it compare to that of more conventional therapies/products/techniques.
* Have you had much interaction with (EMA, MHRA, HTA, HFEA) – how useful have they been?
* Are there any ‘chain of custody’ or accountability issues that arise with biological materials (including viral vectors)?
* Have you had to licence any IP in order to do your research? (again we do **not** need details of specific licenses)
* Do your foresee challenges in terms of commissioning & reimbursement? (especially with regards to the NHS)
* Are hospital ‘specials’ or compassionate use exemptions a useful potential option for gene therapies?

7) **Perspective and future of the field**

* How do you see the UK’s position in relation to the wider global work on gene therapy (and gene editing if relevant)?
  + Do you see the UK as a leader in any particular aspect gene therapy research and commercialisation?
  + If so which?
  + Who are the UK’s main competitors?
  + What are your views on the institutional changes/initiatives that have been made/launched to accommodate cell and gene therapy in the Uk (e.g. the UK Regenerative Medicine Platform, the cell and Gene Therapy catapult)?
* How do you see gene therapy being used in the short term?
  + Which areas of research do you think are the most important to take forward – which applications deserve the most support?
  + Which diseases/conditions, if any, should be priority targets? Why
  + How do you see things developing in the longer term, over the next few decades?
* Do you envisage future clinical applications of CRISPR/cas9 and similar tools?
  + As wholly novel products?
  + As ‘second/ third generation cell and gene therapies’?
  + If not, can you say why?
* What, if any obstacles do you envisage for future gene therapy applications?
  + Scientific?
  + Cultural/Social?
  + Economic (including access to financing / venture capital)?
  + Legal/Regulatory?
  + Manufacturing/ scale up