

Axovia Therapeutics: Q&A Information Sheet

1. When will clinical trials begin in humans with BBS?

AT: We do not have a firm timeline yet, although we are working toward the first human trials as fast as possible.

BBS UK: It is our understanding that the target for the first clinical trials is towards the end of 2022.

2. Who will be in the clinical trials? How do we get involved?

AT: When we get near to conducting clinical trials, we will work closely with the BBS community to keep everyone updated. We are targeting the first clinical trials for end 2022.

BBS UK: The clinical trials will initially focus on the BBS1 gene, however, we do not yet know what the criteria will be for inclusion or how participants will be identified. BBS UK will follow developments closely and ensure the BBS community are kept updated.

3. Can I have gene therapy if I have clinical diagnosis?

AT: The first gene therapy that Axovia Therapeutics is developing will be for BBS1 patients.

BBS UK: No. Gene therapy targets specific genes, which means that a genetic diagnosis is needed.

4. The main research seems to be in BBS1, will there be research for those with BBS10, BBS12 and other subsets?

AT: We are focused on BBS1 initially, and will advise as we expand our targets.

BBS UK: It is our hope that the research will be extended to include further subsets of BBS, however, which genes are selected next isn't yet clear.

5. How long will it take for the research to become a treatment that benefits everyone with BBS?

AT: We are focused on BBS1, and we are working on accelerating the development of gene therapy treatments. It is hard to predict exactly when we will have those available to patients but we will keep the BBS UK community posted on our progress.

BBS UK: Taking research forward into treatments can be a long process dependent on many unpredictable variables. We will keep the BBS community updated on progress via our website, social media, conference and newsletters. We will also develop information resources to help our community understand the process.

6. Will gene therapy stop children with BBS from going blind?

AT: This is the ultimate goal for our first planned treatment for BBS1.

7. Will gene therapy help older people who have already lost a lot of their vision?

AT: We are still in the early exploratory stages of BBS1 gene therapy development and hope to target young and adult patients alike.

8. Will gene therapy treat other aspects of BBS like obesity and kidney failure? Will this be in all age groups?

AT: We expect to treat obesity, we are working on developing a treatment for the kidney and other organs in the future.

9. What about autism and intellectual disability? Will gene therapy help with those too?

AT: Our hope is that our treatments can be beneficial in these areas too.

10. Will eventual treatments be available on the NHS or through BBS clinics?

AT: This is our hope that our treatments will be available on the NHS in the future.

11. Will Professor Beales still be involved in my care at the BBS clinics?

AT: Yes.