



Patients' Views on Genome Editing

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ACKNOWLEDGEMENTS

Thank you to all the people living with rare and genetic conditions that have contributed to Genetic Alliance UK's work on genome editing.

Thank you to EURORDIS – Rare Diseases Europe, and to Progress Educational Trust for collaborating with Genetic Alliance UK on this topic.



PATIENT PERSPECTIVE

November 2016

Following a series of education webinars, patients affected by a rare genetic condition:

- welcome the use of genome editing technologies in research and clinical settings
- are interested in genome editing technologies, and would like to learn more about them
- are clear that use of the technology in humans should be limited to treating medical conditions
- believe the benefits of this new and innovative technology far outweigh potential risks;
- want a multiple stakeholder approach to regulatory decisions to ensure ethical use of genome editing technologies, and want to be involved in this process

Full report: geneticalliance.org.uk/wp-content/uploads/2016/05/nerri_finalreport15112016.pdf

GENOME EDITING TECHNOLOGIES

The patient perspective



BASIC UNDERSTANDING OF GENOME EDITING

September 2017 – A collaboration between Genetic Alliance UK and Progress Educational Trust, funded by Wellcome.



Eighteen patients and carers affected by genetic conditions and fourteen fertility sector patients and practitioners attended a series of five day-long workshops.

Eight recommendations aimed at ensuring clarity of communication about genome editing.

Full report: geneticalliance.org.uk/our-work/medical-research/understanding-genome-editing/



RATIONALE

Definition is crucial to progress understanding and work towards common ground.

- Big data
- Personalised medicine
- Mitochondrial replacement therapy

Genome editing is an incredibly broad topic: technique, treatment, research tool etc. A broad public discussion on genome editing will help us to realise the potential of genome editing, and ensure that the public have a say in the limits of its use.

On complex topics naive viewpoints are not useful:

The valuable patient view is the view of an informed patient.

Full report: geneticalliance.org.uk/our-work/medical-research/understanding-genome-editing/



OUR COMMUNITY IS STRONGLY IN SUPPORT OF EXPLORING THE POSSIBILITIES OF GENOME EDITING

The bottom line following two projects on the topic

'I feel any person with a life changing or life limiting condition should be given the chance of a normal life'

'If it was for the greater good, then yes I would be pleased to help this research'

Survey respondents **Genome Editing: What does it mean for patients? (2016)**

UK patients and carers living with rare and genetic conditions expect that genome editing would

- accelerate research
- deliver information
- lead to a better understanding of genetic conditions

They expressed some wariness in light of claims previously made for gene therapy, and in light of the unfulfilled promise of cures for conditions such as thalassaemia.

[Understanding Genome Editing – Genetic Alliance UK and Progress Educational Trust \(2017\)](#)



PERSPECTIVES FROM RARE DISEASE POLICY EXPERIENCE

Are all stakeholders equal here?

- Who stands to benefit most from innovation?
- Who takes the risks when trialing new therapies?

Patients, carers, families with unmet health needs are the ultimate stakeholder. Their experience should frame this debate.



THANK YOU

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