

The impact of rare disease



7000+

rare diseases, many life threatening.

3.5 million

affected in the UK alone



70%

of cases affect children.

95%

lack effective treatment.



What is ARDAT?

- Founded in 2019, funded by Innovative Health Initiative
- A consortium of 32 partners from academia, industry & SMEs worldwide
- Our mission is to advance our collective knowledge about ATMPs and accelerate the development of new treatments for rare diseases.



innovative medicines initiative



Aims:

Understand immune responses to ATMPs



Advance AAV delivery technology



Create a shared research biobank



Harmonise global regulations



Achievements:

Developed 3D liver & CNS immune models

Improved AAV efficiency & gene delivery

Established the ARDAT Biobank (REC approved)

Engaged with key global regulators

We've also...



Released 13 publications, several others under review



Progressed a new gene therapy



Listened to those with lived experience