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# Rare Treatment Accelerator

Unlock the power of repurposed drugs for rare diseases

## These are the questions in the eligibility questionnaire:

1. Which therapy area is your repurposing drug aiming to treat?
  - a. What is the main rare disease indication you are developing your repurposing drug for?
2. What is the worldwide prevalence and/ or incidence of your main indication?
3. Is your repurposing candidate a monotherapy or a combination?
  - a. Has it been approved in one or more regulatory jurisdiction(s) for human use?
  - b. For combinations, how many individual drug(s) and/or nutraceutical(s) is your repurposing candidate made up of?
4. What is the drug/nutraceutical monotherapy/combinations that you are repurposing?  
*If you do not wish to disclose the identity of your molecule(s), please indicate the class of drug/nutraceutical, or find some other way to describe the molecule so we have some idea how to evaluate it.*
5. Is your repurposing candidate still being manufactured and sold as a drug or nutraceutical?
  - a. What strategy(ies) would you propose to mitigate off-label prescribing?
  - b. If not, why has your repurposing candidate been discontinued?
6. Please briefly outline your rationale for your repurposing opportunity.
7. Is the molecule that you are repurposing still under patent protection?
8. Is there intellectual property patent protection available or already prosecuted for your invention?
  - a. Where is your patent(s) prosecuted?
9. Do you hold the licensing rights of the patent(s) concerned in this drug repurposing opportunity?
10. Has the natural progression of your targeted rare disease indication(s) been characterised and described in natural history studies?
11. Are there clinical biomarkers and/ or endpoints published/ previously agreed by regulators for your targeted rare disease indication?
12. Do you have *in vivo* data supporting the use of your repurposed drug candidate for your targeted rare disease indication?
  - a. Do you believe you have enough preclinical data to support starting a clinical trial in the next 6 months if there was financing and expertise available to conduct the clinical trial?
  - b. What is the highest phase of clinical development your asset is ready for?
13. Are you prepared to license this technology to industry for development?
14. Are you willing to co-develop this technology with industry through co-investing?
15. Has the opportunity been considered by other parties?