

## FAQs for RFA-TR-20-031

**Question** Do the two diseases have to be from different RDCRN consortia ? Or can are both be from the same RDCRN consortium?

**Answer:** The two diseases can be from the same RDCRN consortium. “If the PD/PI is not involved with the RDCRN, a Letter of Support from the relevant RDCRN investigator needs to be included.”

**Question:** Can the project include more than two diseases ?

**Answer:** Yes. “The purpose of this FOA is to provide support for applications that propose a clinical trial of drug that targets a shared molecular etiology and includes at least two different rare diseases, and in the process to identify and overcome challenges in adapting the oncology basket trial model to rare diseases.”

**Question:** If the UG3 phase focuses on two diseases, but one of them turns out to be unsuitable for the clinical trial, can you proceed to the trial in the UH3 phase with only one disease ?

**Answer:** No, there must be at least two diseases in the clinical trial. “This cooperative agreement will also support the subsequent small clinical trial, involving at least two different rare diseases.”

**Question:** How do we split the 12 page limit between UG3 and UH3? Is there a recommendation?

**Answer:** No, the decision is up to the PI .

**Question:** Should we involve RDCRN investigators for both diseases represented?

**Answer:** You should involve RDCRN investigators for all RDCRN diseases under study.

**Question:** Can hyperammonemia be considered a common etiology — it is a common symptom of 8 urea cycle disorders and several organic acidemias?

**Answer:** It is up to the PI to make the case for a common etiology in the application.

**Question:** If we already have an IND to study a drug in one disease, can we propose a study to conduct studies in this disease as well as a second disease with similar etiology?

**Answer:** Yes, as long as they are rare. “The purpose of this FOA is to provide support for applications that propose a clinical trial of drug that targets a shared molecular etiology and includes at least two different rare diseases, and in the process to identify and overcome challenges in adapting the oncology basket trial model to rare diseases.”

**Questions:** To clarify, we should propose to study 2 diseases both of which are currently studied by RDCRN. Is this correct? And must projects focus ONLY on diseases which are currently under study by the RDCRN, or can they focus on a basket of diseases which INCLUDE some studied by the RDCRN?

**Answer:** Projects must include at least two diseases under study by the RDCRN. If additional diseases are included, they do not all have to be under study by the RDCRN. “To maximize the impact of NIH investments in rare disease clinical research, including the development of clinical endpoints based on

natural history data, projects must focus on diseases which are currently under study by the Rare Disease Clinical Research Network (RDCRN) <https://www.rarediseasesnetwork.org/diseases#FAD2>.”

**Question:** Does the direct budget have to cover indirect costs for sub-contract sites for the trial?

**Answer:** The parent (Main) site will need to ensure that both direct and indirect costs for the consortium sites are included in the Direct Cost consortium line item, however the consortium F&A is excluded from the direct cost limit (UG3: \$350,000 and UH3: \$600,000) in both phases.