
Oligonucleotide Toxicity
Open Data Challenge
OligoTox Challenge
NIH
National Center
for Advancing
Translational Sciences

# Agenda

OligoTox Challenge Goals and Background
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Q&A

NCATS Staff

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# OligoTox Challenge Background

**Oligonucleotide** (“oligos”) therapeutics are an emerging therapeutic modality with increasing numbers of drugs in development and several approved for use in the treatment of rare genetic diseases.

Both the FDA and NIH are interested in **new approach methodologies** that can reduce the use of animal testing in preclinical safety studies such as through technologies like organoids, which are small, lab-grown tissue models that replicate the structure and function of human organs.

In recent years, we have also witnessed dramatic increases in the **use of advanced computational methods** to address complex biological problems.

# OligoTox Challenge Background (cont.)

The Challenge: NCATS seeks to incentivize the generation of **high-quality and publicly accessible open datasets** that make use of in vitro human systems and can be used to advance predictive models for oligo toxicity.

Toxicities of particular interest include hepatotoxicity, nephrotoxicity, thrombocytopenia, complement activation, coagulopathy, immunotoxicity, chronic neurotoxicity, and hydrocephalus.

# Schedule, Prizes, Submission

## Phase 1: **Ideation [Ended]**

Start: **December 19, 2025**
Submission Deadline: **February 28, 2026**

Aim: **Propose how you will collect, build, or a contribute dataset from in vitro human-based systems that can be used to support the development and training of in silico models to accurately predict toxicity for a candidate oligo therapeutic**

Prizes: **Maximum of ten winners with a prize of up to $10,000 each**

## Phase 2: **Data Generation**

Start: **May 1, 2026**
Submission Deadline: **December 31, 2026**

Aim: **Make publicly available a high-quality open dataset with demonstratable use in developing and training predictive models for oligonucleotide toxicity**

Prizes: **Maximum of two winners with a prize of up to $100,000 each and four runners-up with a prize of up to $50,000 each**

# Phase 2 Submission Requirements

Four parts: a **narrative document**, a **methodology document**, a **public access and dissemination plan**, and a **dataset** (see listing for details).

1. **Narrative document**
   - An executive summary of the dataset(s) generated, and positive/negative controls included
   - A summary of the main findings and conclusions
   - A description of how data were produced, including descriptions of relevant experimental design, data acquisition and any computational processing of raw data
   - A description of how indicators and predictor variables for oligo toxicity were measured, their distribution, and the distribution of predictor variables amongst tested oligos
   - A discussion of how the results address a gap in the publicly available data relating to one or more aspects of oligo toxicity
   - A discussion of how the data could be used to develop a predictive model of one or more aspects of oligo toxicity

# Phase 2 Submission Requirements (cont.)

Four parts: a **narrative document**, a **methodology document**, a **public access and dissemination plan**, and a **dataset** (see listing for details).

2. **Methodology document**
   - Information on the materials and methods used to generate the data
   - This should include the methods used to purify and characterize oligo identity.
3. **Public access and dissemination plan**
   - Describes how winners will disseminate information about their solution and make their solution, as well as the knowledge necessary to utilize their datasets, openly available for research purposes to the public
   - Any terms for data access and data use should be defined in allowing for open and public access, such as through a creative commons license or other licensing scheme
4. **Dataset**
   - Access to the raw data
   - Data dictionary and schema documenting all metadata
   - Should include sequences of all oligos tested, as well as the location of all chemical modifications in each oligo, data on the purity and characterization of each, and any additional metadata

# Evaluation and Judging

Entries that comply with eligibility and submission requirements will be scored for scientific value, experimental approach and design, data translatability, and proposed dataset management.

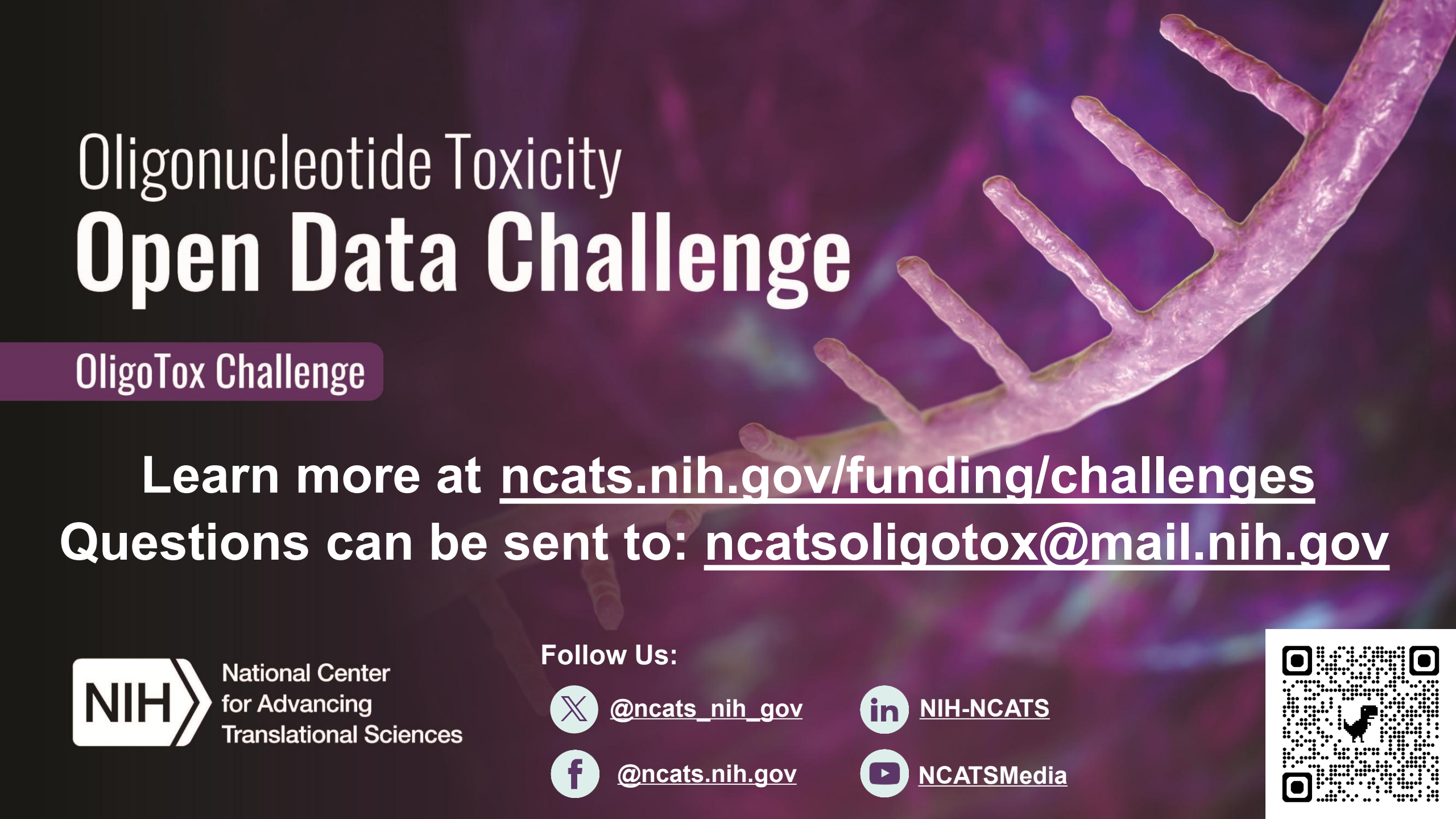
A **Judging Panel**, that will include Federal employees from NIH and other government agencies, as well as non-Federal subject matter experts will determine winners by assigning confidence ratings on how well submitters address these **four factors**:

- **Ability to Solve the Challenge -** Appropriateness in advancing predictive modeling of oligo toxicity
- **Potential Impact -** Ability for the dataset to address important information gap(s)
- **Feasibility and Rigor -** Viability and quality of this dataset
- **Transparency and Reproducibility** – Adherence to NIH scientific data sharing policies and best practices

# Q&A

Important Dates:

- Phase 2 Submission Ends: **December 31, 2026**
- Phase 2 Winner(s) Announced : March 15, 2027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