

ICARE Member Reports

- NIH Office of Rare Diseases Research
- The Office of Rare Diseases Research (ORDR) was established in 1993 within the Office of the Director of the National Institutes of Health (NIH), the Federal focal point for biomedical research. ORDR coordinates and supports rare diseases research, responds to research opportunities for rare diseases, and provides information on rare diseases. Public Law 107-280, the Rare Diseases Act of 2002, established the ORDR by statute.
- The goals of ORDR are to stimulate and coordinate research on rare diseases and to support research to respond to the needs of patients who have any one of the more than 6,800 rare diseases known today.

ICARE Member Reports: ORDR

Types of research support or other activities

- Total office budget of about \$18M – no grant making authority requires working with Institute and patient group partners
- Rare Disease Clinical Research Network – approx. \$11.5M/yr.
19 consortia plus data management center
~100 diseases represented/~60 patient support groups affiliated with Network
- NeuroMab – ORDR contributed \$ for rare disease-specific projects
- Bench-to-Bedside – integrates the work of basic and clinical scientists at NIH and between intramural and extramural researchers – approx. \$1M/yr.
- Conferences – co-sponsor approximately 100 conferences per year – approx. \$1M/yr.
- Undiagnosed Diseases Program – launched last yr. Looking for new diseases and rare variants of more common diseases – approx. \$1.75M/yr.
- Curriculum supplement – creating a middle school science curriculum supplement to demystify and destigmatize rare diseases
- Genetic & Rare Disease Information Center (GARD) – approx. \$1M/yr.

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Resources available for shared use or other opportunities for collaboration

- **Parallel, but hopefully complementary, efforts**
- Therapeutics for Rare and Neglected Diseases (TRND)
 - similar to Blueprint
 - resources to overcome the “valley of death”
- Registries and Biobanks – working to develop standards as well as a library of common data elements, questions, etc.
- CETT – development of genetic testing from research lab to clinical practice, along with the appropriate educational materials – approx. \$1M/yr.
- Clinical Trial design – various efforts including
 - studies within RDCRN
 - adaptive clinical trial design
 - course w/FDA on science of small trials (avail on NIH videocast)
- Institute of Medicine review – currently ongoing – ORDR plus FDA’s OOPD
 - will recommend new ways for ORDR to get involved in rare diseases research activities