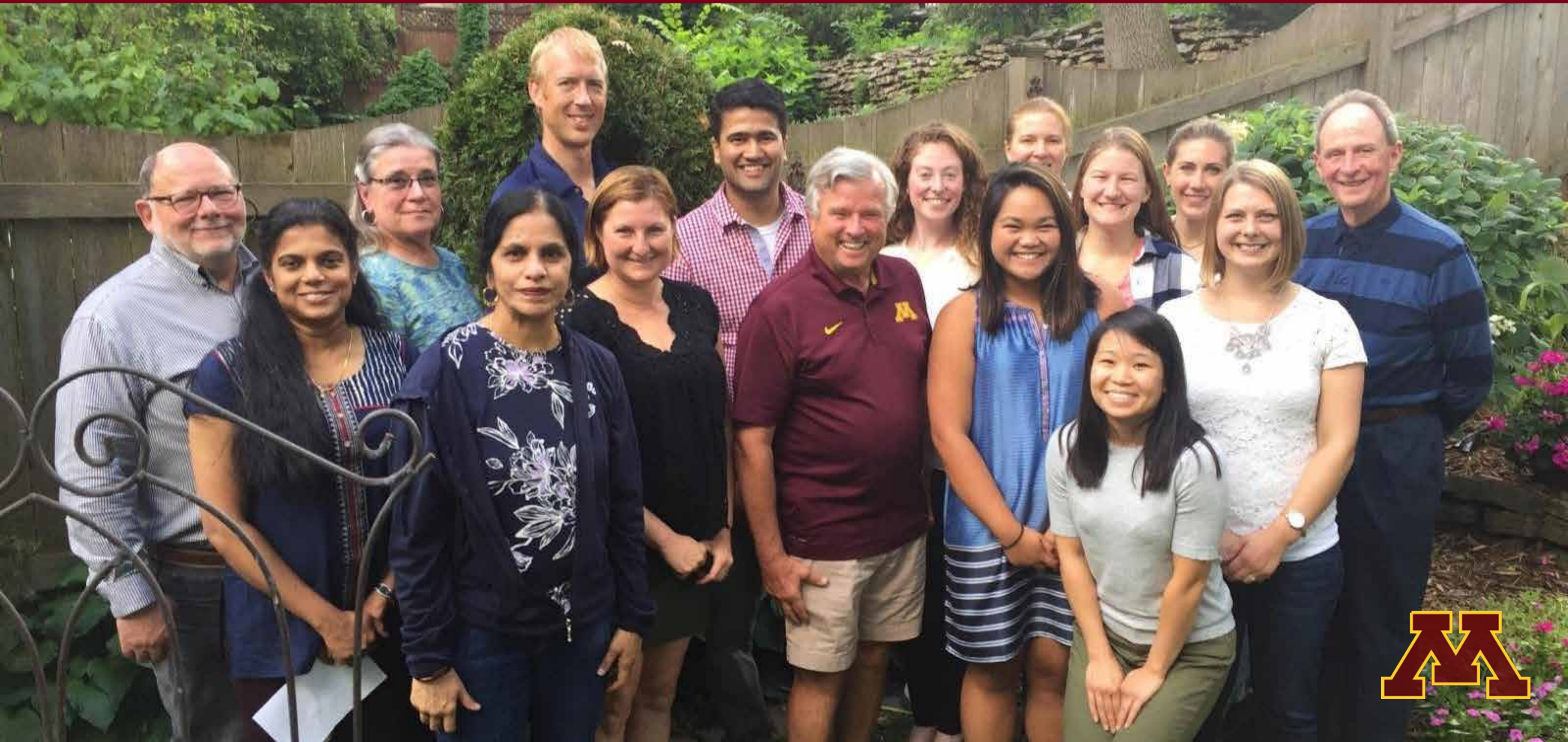
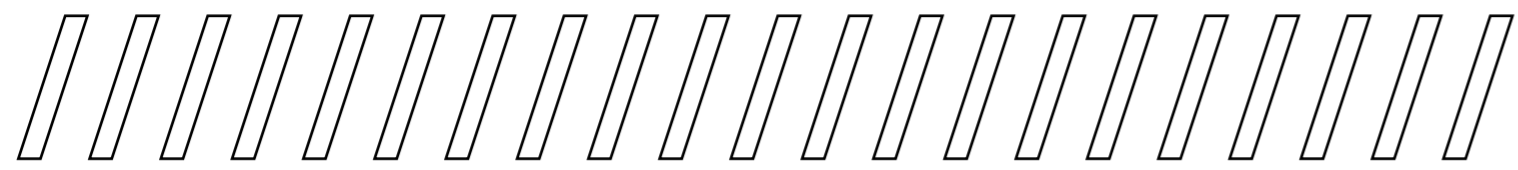


Center for Orphan Drug Research 2015-17 Report





The Center which is housed in the McGuire Translational Research Facility was established in 2005. Initial efforts were focused on continuation of epilepsy research. In 2007, a formal name was selected, Center for Orphan Drug Research (CODR), and a mission statement was adopted:

The mission of the Center for Orphan Drug Research (CODR) is to improve the care of individuals who have rare diseases through research on new drug therapies; education of health professionals and health profession students; and contributions to the discussion and formulation of public policy relating to rare diseases and orphan drugs. The focus of CODR research is rare pediatric neurological disorders.

In the ensuing 10 years, the Center has added research staff; grown our research portfolio; entered into a wide range of interdisciplinary, inter-collegiate collaborations; expanded membership; attracted graduate, professional, and undergraduate students; added a post-doctoral fellowship; and developed and maintained an active outreach/advocacy program.

The following is a summary of the Center's accomplishments over the last 2 years. Full details about our activities are found in the attached appendices.

Center Overview

CONTENTS:

RESEARCH

EDUCATION

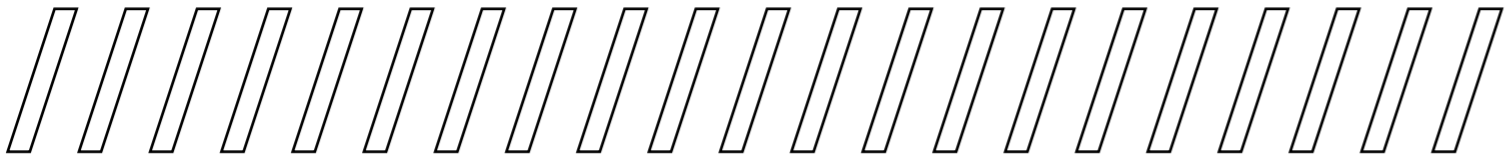
HONORS & AWARDS

FINANCIAL OVERVIEW

APPENDICES



Research



Research

The Center has enjoyed substantial growth over the last 2 years as we have expanded our portfolio in all three focus areas: epilepsy and seizure disorders, spasticity, and neurodegenerative disorders. The impending receipt of a NOGA* for an RO1 (Coles, PI) supporting a 5 year study of the pharmacokinetics and pharmacodynamics of the 3 drugs used in the Established Status Epilepticus Treatment Trial will further add to our research program. The Center continued expanding its collaborations within and outside the University.

26
proposals were submitted to various sponsors



1
orphan designation was approved, 1 is pending

18
proposals were ultimately funded for a total of \$1,981,532.00 from various sponsors



3
patents were approved



3
INDs were approved

23
articles were authored and published in various publications



Orphan destinations: IV TPM and IV Baclofen (pending)
Approved patents: Development of water-soluble benzodiazepines activated by enzymes for use as intranasal seizure rescue, IV carbamazepine, and IV topiramate

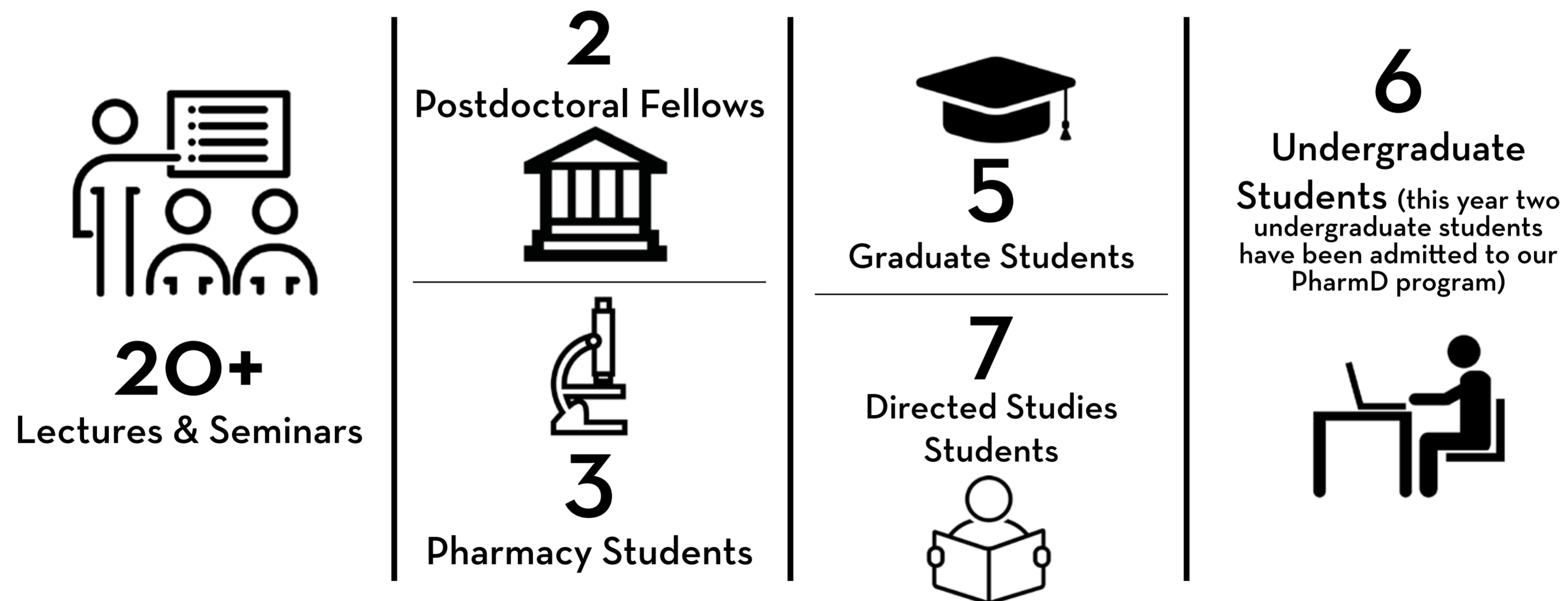
* This award was received by the Center on 8/4/2017.

Education



Education

The Center makes a substantial commitment to all levels of University education. The Center has attracted a number of outstanding trainees who participate in our various research projects. Many of these students have received support from Clinical and Translational Science Institute (CTSI) and Undergraduate Research Opportunities Program (UROP). Several have gone on to pharmacy school.



Outreach



Outreach/Advocacy

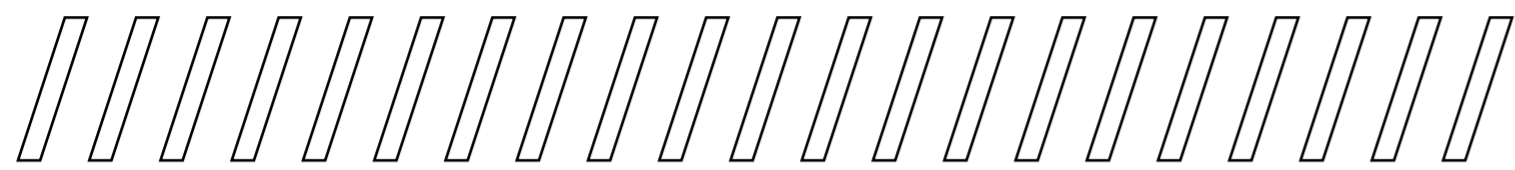
Rare Disease Day: The Center annually co-sponsors Rare Disease Day. The most recent event occurred on 2/24/2017 in the McNamara Alumni Center. The event attracted 150 attendees, 16 Patient Advocacy Groups, and 30 research posters. The keynote speaker was Steve Groft, PharmD, Senior Advisor to the Director, National Center for Advancing Translational Sciences, NIH.

The Center participates in multiple other advocacy activities throughout the year including Feed My Starving Children, Rotary Club lecture, University of Minnesota's Graduate School Research Showcase, the Epilepsy Foundation's Walk for Epilepsy, and the Anita Kaufmann Foundation's Walk for Epilepsy and Guinness World Record Largest Epilepsy Training Session.



RARE DISEASE DAY®





Honors & Awards

The students of the Center have received a number of honors and awards over the past 2 years:



Bridget Curtin, BS

Accepted into the University of Minnesota College of Pharmacy PharmD program
2016 CTSI Pathways to Research Program scholar
2017 CTSI Pathways to Research Program scholar
2016 CTSI Pathways to Research Program best poster travel award
2016 Undergraduate Research Opportunity Program award

Ghada Elnashar, BS

2015 Melendy Fellowship



David Kraus, BS

2016 UMN Department of Chemistry Senior Award



Patricia Maglalang, BS

Accepted into the University of Minnesota College of Pharmacy PharmD program
2015 CTSI Summer Research Scholar
2015 Center for Health Equity Research Scholar
2nd Place Best Student Poster Award Winner, Institute for Engineering and Medicine Annual Conference
2016 UMN Department of Chemistry Senior Award



Abhishek Sathe, PhD student

2016 James C. Cloyd Neuropharmacology Research Fellowship



Natalie Schmitz, PharmD, MPA, PhD student

2017 Zasko Fellowship
2016 James C. Cloyd Neuropharmacology Fellowship
2015 CTSI Advanced Research Scholar



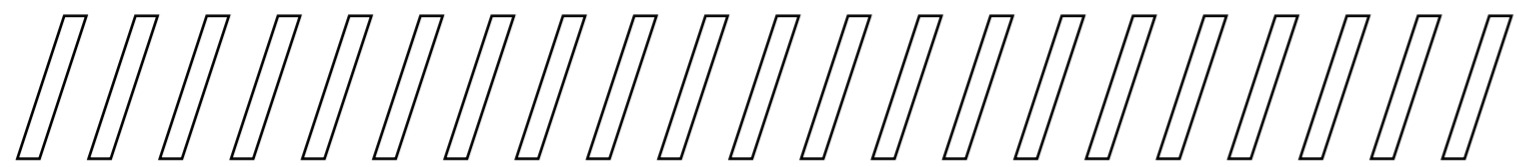
Irene Vuu, PharmD, PhD student

2017-2018 American Epilepsy Society Pre-Doctoral Fellowship
2017-2018 American Foundation of Pharmaceutical Education Pre-Doctoral Fellowship
2016 American Epilepsy Society Fellows Program
2016 American Neurological Association Translational and Clinical Research Course for Clinician-Scientists

Kwangchae Yoon, PharmD, Fellow

2016-2017 Genzyme LDN Fellowship





Honors & Awards

The faculty and staff of the Center have received a number of honors and awards over the past 2 years:



James Cloyd III, PharmD

2015 Rho Chi Lecture Award, Rho Chi Society
2015 Sumner J. Yaffe Lifetime Achievement Award in Pediatric Pharmacology and Therapeutics, Pharmacy Pediatric Advocacy Group
8th Annual Denis J. Crankshaw BioPharm Lectureship, McMaster University, Hamilton, Ontario
Invited Lectureship: 60th Annual Julius Koch Lecture, School of Pharmacy, University of Pittsburgh
Outstanding Advising and Mentoring Award, awarded by the UMN Graduate and Professional Student Association
2016 UMN CTSI Mentor of the Year



Lisa Coles, MS, PhD

2015 Anti-epileptic Drug Development Fellowship
2016 Junior Mentor of the Year



Reena Kartha, MS, PhD

2016-17 Rare Disease Clinical Research Network Clinical Research Certificate Program Scholar
2016-17 Lysosomal Disease Network Clinical Fellowship

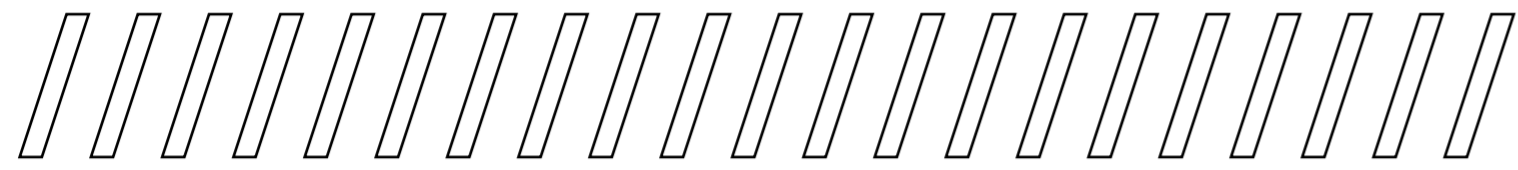


Usha Mishra, MS

2017 Elected to Board of Directors for Minnesota Mass Spectrometry Society

Marcia Terluk, PhD

2017 Certified Phlebotomist

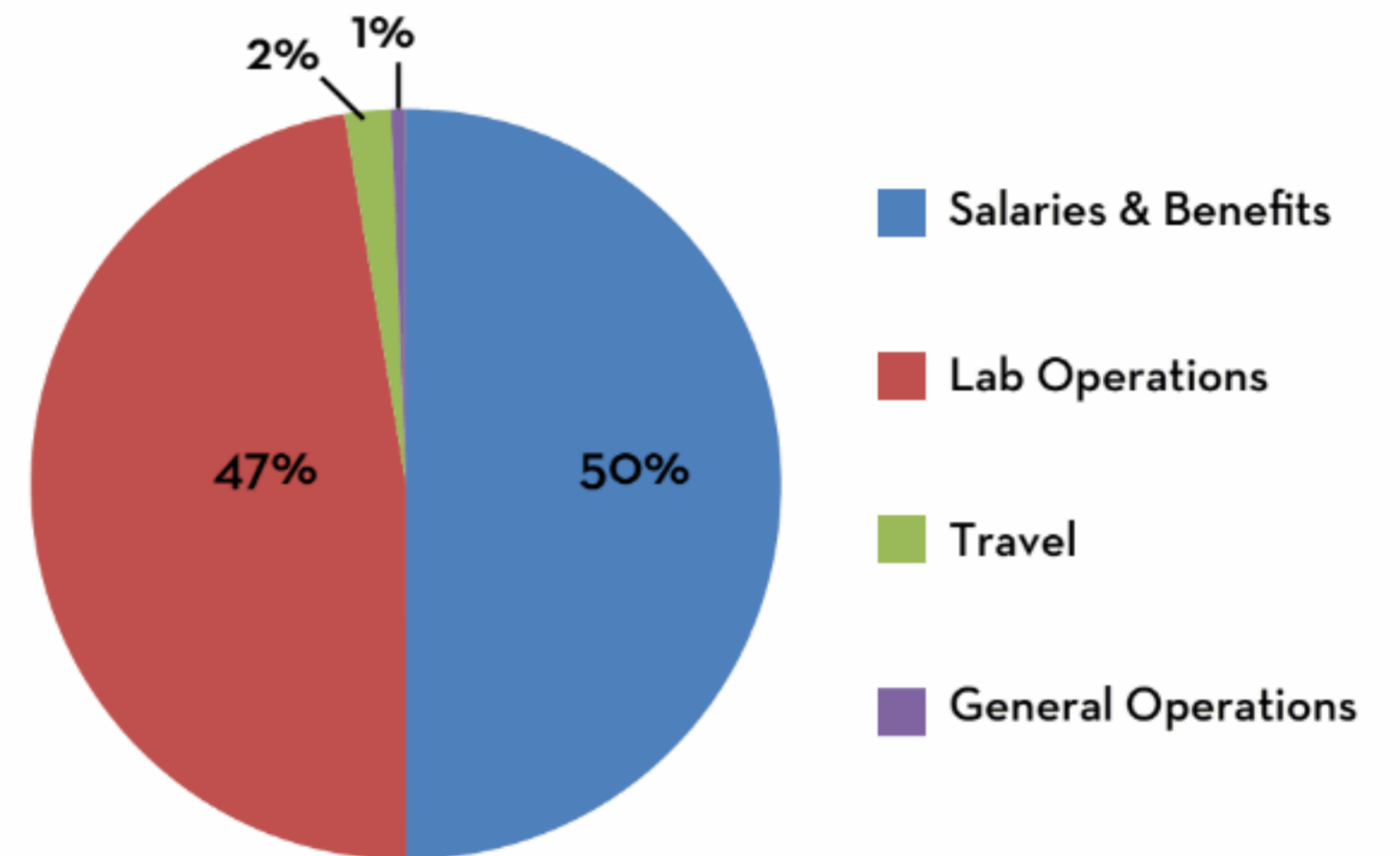
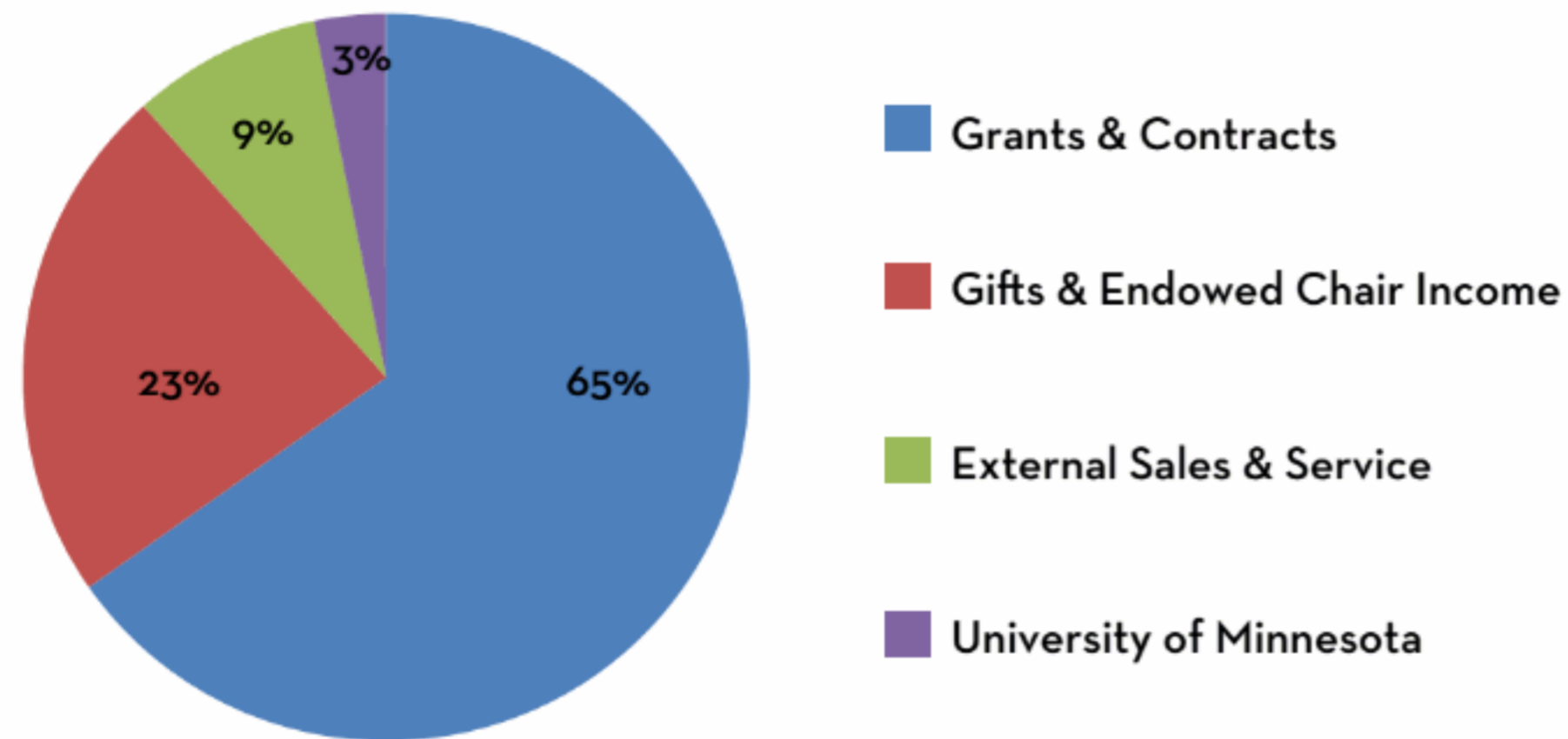


Financial Overview

7/1/16-6/30/17

REVENUE SOURCES FOR FISCAL YEAR 2017

EXPENSES FOR FISCAL YEAR 2017



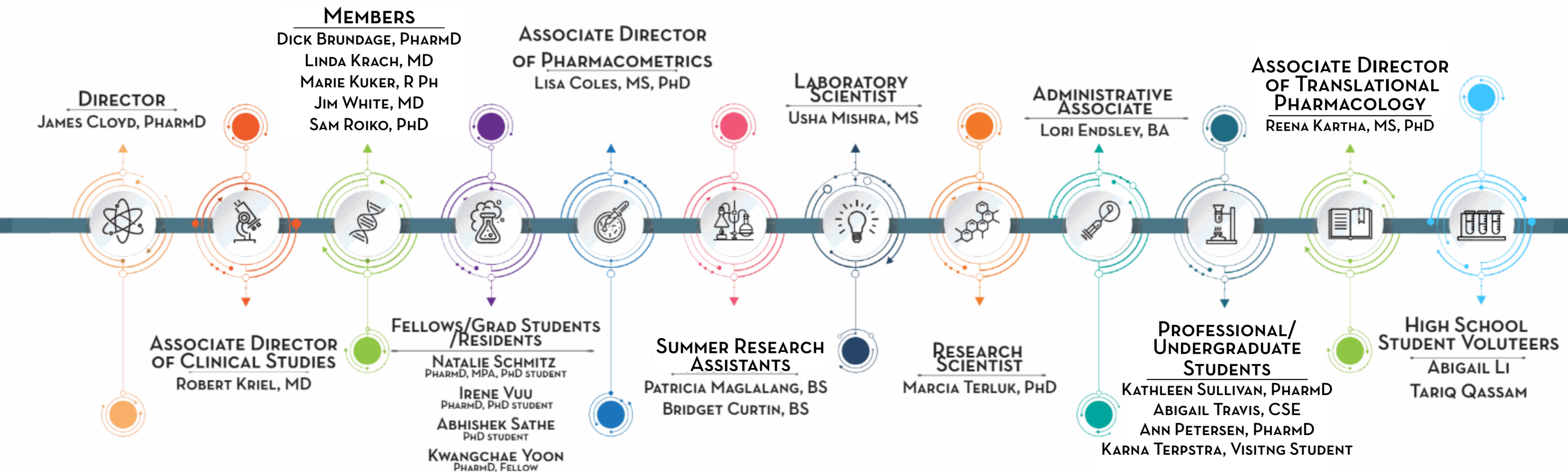
Personnel

NEW HIRES/APPOINTMENTS:

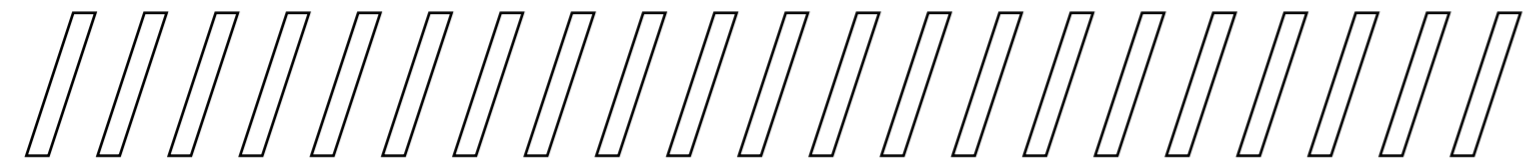
Jim White, MD (Minnesota Epilepsy Group) and Sam Roiko, PhD (Gillette Specialty Healthcare) were welcomed as new CODR members.

Marcia Terluk, PhD joined the Center as a Research Scientist.

CURRENT ROSTER



Appendices



- 2015-2017 Submitted proposals
- 2015-2017 Peer-reviewed publications
- 2015-2017 Presentations
- 2015-2017 Research projects
- 2015-2017 Education
- 2015-2017 Additional activities and accomplishments
- FY17 Financial Overview

