

# **Center for Orphan Drug Research (CODR)**

**College of Pharmacy  
University of Minnesota**

**Reena Kartha, MS, PhD  
Jim Cloyd, PharmD**



# Rare Diseases & Orphan Drugs

## Rare Diseases

- Conditions affecting a small number of people
  - US FDA = prevalence of less than 200,000 (1 in 1650)
  - European Union = affects fewer than 1 in 2,000 people
- An estimated 25-30 million (1/11) in US (NIH)
- Majority (~2/3) affect children and 80% have a genetic origin
- Rare disorders are often devastating, life-threatening and difficult to diagnose
- ~7000+ distinct rare conditions (NIH) (and the # is growing)

## Orphan Drugs

- Medications used to treat rare disease
- Only 5% of rare diseases have an approved therapy
- The Orphan Drug Act (1983) has resulted in ↑ # of FDA-approvals of orphan drugs: 26 of 52 novel drugs in 2021

# Center for Orphan Drug Research (CODR)

Established-2005

College of Pharmacy, University of Minnesota

**Mission:** To improve the care of individuals who have rare pediatric neurological disorders through **research** on new drug therapies; **education** of health professionals; and **community engagement and advocacy** relating to rare diseases and orphan drugs.



McGuire Translational Research Facility

Member of the University of Minnesota Rare Disease Center of Excellence  
<https://qa-cm.mhealthfairview.org/rare-diseases>

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# **CODR Personnel**

**Director:** Jim Cloyd, PharmD

## **Associate Directors**

**Clinical Research:** Robert Kriel, MD (Pediatric Neurology)

**Translational Pharmacology:** Reena Kartha, MS, PhD

**Clin Pharm/Pharmacometrics:** Lisa Coles, MS, PhD

## **Members**

**Dick Brundage, PharmD, PhD (Pharmacometrics)**

**Linda Krach, MD (Pediatric Rehab Medicine)**

**Jim White, MD (Adult Neurology)**

**Marie Kuker, RPh (Regulatory)**

**Sam Roiko, PhD (Pediatric Clinical Pharmacology)**

**Erica Barnes, MS (Ex Director, MN Rare Disease Advisory Council)**

**Laboratory Scientist:** Usha Mishra, MS

**Research Scientist:** Marcia Terluk, PhD

**Postdoctoral Fellow:** Jaehyeok Roh, PharmD

**ECP Administrator:** Lori Endsley

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# CODR Research



# CODR Areas of Research

## Epilepsy Syndromes

Infantile spasms  
Neonatal seizures  
Antiepileptogenesis  
Seizure emergencies

## Spasticity

Baclofen  
withdrawal

## Neurologic/Psychiatric Disorders

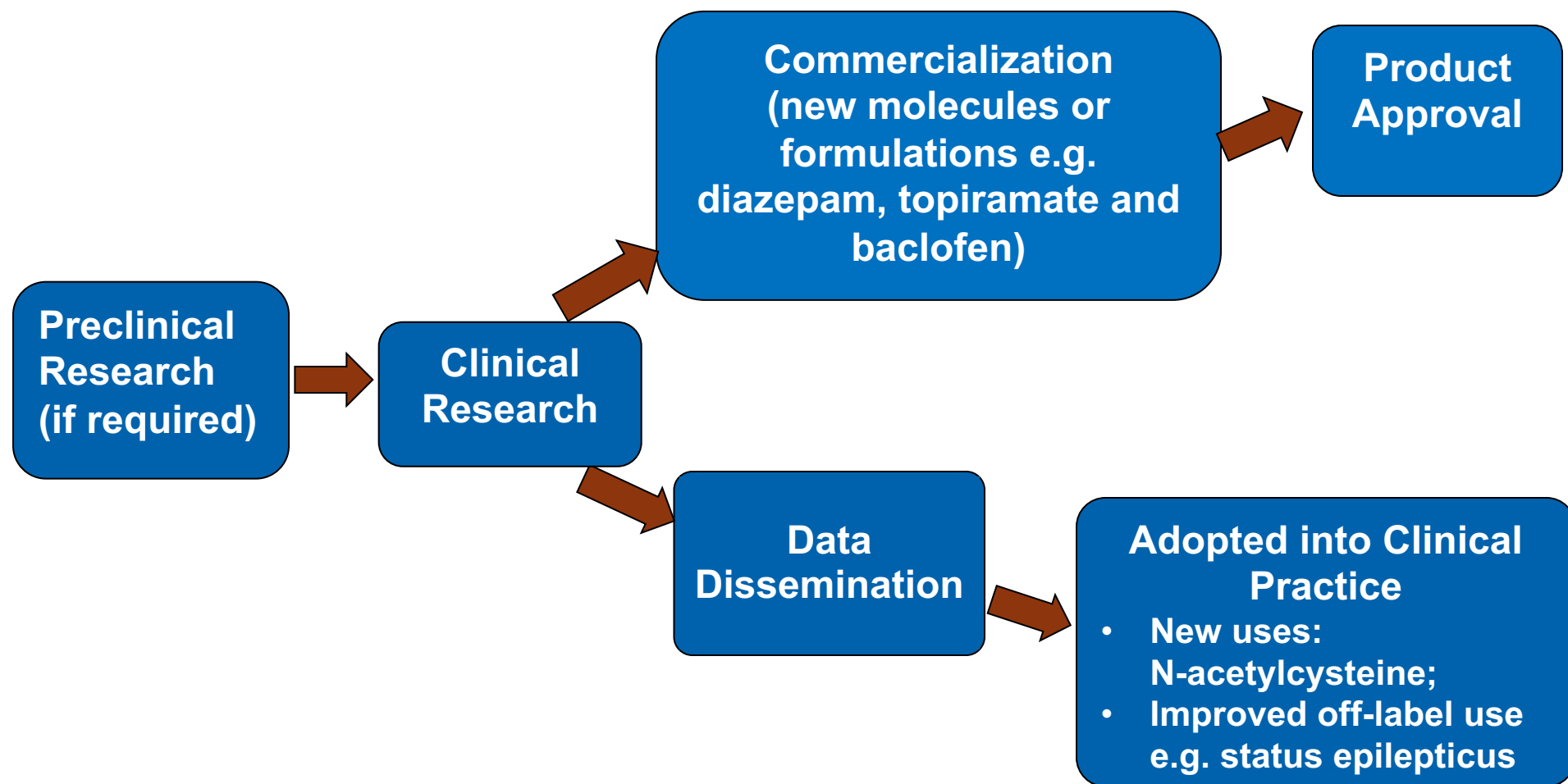
Adrenoleukodystrophy  
Gaucher Disease  
Parkinson's Disease  
Non-suicidal Self Injury

### Research Tools (humans & animals)

### Models

- Mechanisms of Action
- Biomarkers
- Formulations
- Pharmacokinetics & Metabolism
- Safety and Efficacy Studies
- Pharmacometrics
- *In-vitro* (established cell lines)
- *Ex-vivo* (patient-derived primary cells)
- *Animals* (disease-specific)

# CODR Model of Orphan Drug Development



# CODR Research-Approved Products & Drugs Under Development

Rare Disorder	Drug	Status
Seizure clusters	Rectal Diazepam*	Approved
Seizure clusters	Intranasal diazepam prodrug	Pre-clinical studies
Status epilepticus	Intravenous allopregnanolone	Pre-clinical
Bridge therapy and neonatal seizures	Intravenous topiramate*	Phase I healthy volunteer study
Spasticity	Intravenous baclofen*	NDA submitted
Gaucher disease Type I	Oral N-acetylcysteine	Studies in healthy volunteers and patients
Neuronopathic Gaucher disease	Oral N-acetylcysteine amide	Pre-clinical studies using patient-derived fibroblast cells
Adrenal insufficiency.	Rescue Therapy	Pre-clinical studies
Non-suicidal self-injury**.	Oral N-acetylcysteine	Studies in patients
Adrenoleukodystrophy	Nervonic acid	Pre-clinical studies

\*Designated by the FDA as an orphan product \*\*not a rare disorder



# CODR Education



# **CODR Education Activities**

- **Presentations at National and International Conferences**
  - **National Organization of Rare Disorders**
  - **European Infrastructure for Translational Medicine**
  - **Indian Society for Inborn Errors of Metabolism**
  - **American College of Clinical Pharmacy**
  - **FDA Workshops on Orphan Designation**
  - **Gaucher Community Alliance**
- **Center for Orphan Drug Research Weekly Seminars**
- **Lectures-Pharmacy students, Graduate Students**  
**Medical School, Healthcare Professionals**
- **Research Clerkships**
  - **Pharmacy and Graduate Students**
- **University of Minnesota Undergraduate Course**

# RARE DISEASES: WHAT IT TAKES TO BE A MEDICAL ORPHAN

## Fall 2022, 3 Credits

### Course Director: Reena Kartha, MS, PhD



## Grand Challenge Curriculum

3 credit courses • Open to all students • Meet Liberal Education theme requirements

[Home](#)[About](#)[Courses](#)[Freshman Seminars](#)[C2C Workshop](#)[Student Stories](#)[For Faculty](#) ▾[» GCC 1906](#)

## GCC 1906

GCC 1906

### Rare Disease: What it Takes to Be a Medical Orphan

### Rare Diseases: What it Takes to Be a Medical Orphan

What if you are born with a condition, which very few people know about and for which there are no cures? Or what if there is a cure, but it is very expensive and you have to take it throughout your

### Course Details

**Meeting Time:** Spring 2020,TBD

**Location:** East Bank

**Topics:** economics, public policy, healthcare, business, pharmaceuticals

**Credits:** 3

**Prerequisites:** Freshman or first-year

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# **Community Engagement and Advocacy**

**Interactions with Patient Advocacy Organizations**  
**Annual Rare Disease Day Program**  
**Rare Disease Play**



# Rare Disease Day Programs

**Co-sponsors: UMN Stem Cell Institute and CODR**

## Past Program Themes

- Gene Therapy
- Telehealth
- Care Transition from Childhood to Adulthood
- Patient-focused Drug Development

Help support our research on rare pediatric neurological disorders.

**MAKE A GIFT! ▶**

**SAVE THE DATE**  
RARE DISEASE DAY, FEBRUARY 3/2/2023

**3/2/2023, McNamara Alumni Center  
University of Minnesota**



Round table discussions at the Patient Advocacy Group Breakfast.

<https://www.pharmacy.umn.edu/centers-and-institutes/center-orphan-drug-research>

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# Community Engagement and Advocacy

## Rare: Stories of Dis-ease

The production and performance of a play done through a collaboration among the University of Minnesota Department of Theater Arts and Dance, CODR, and the Sod House Theater

Director: Luverne Seifert  
Dramaturg: Sonja Kuftinec  
Playwright: Kevin Kling

