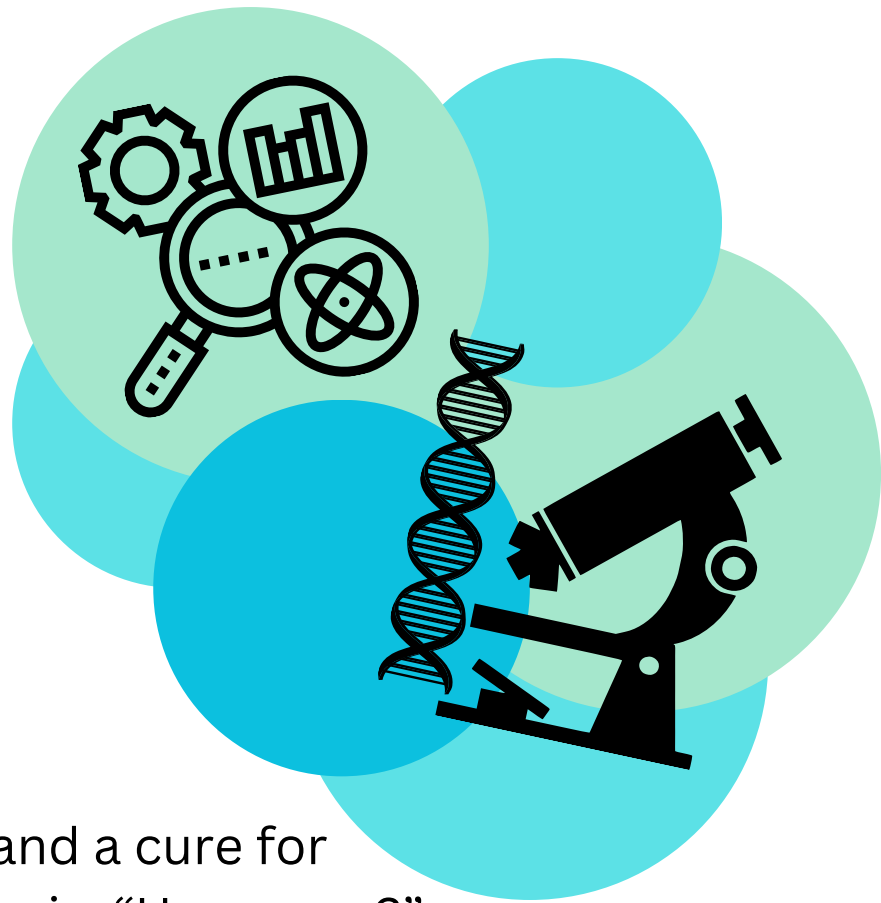




# PPP3CA Hope Foundation: JOURNEY TO A CURE



There will be a treatment and a cure for PPP3CA - the only question is, “How soon?”

Below outlines our research plan over the next 5-10 years. Your donation will directly fund these efforts.

## 1- Build PPP3CA Models

- Identify patients with PPP3CA variants
- Create mice, yeast, zebrafish and stem cells with these PPP3CA variants

## 2- Study these Models

- Understand the impact of PPP3CA mutations on the brain and body
- Learn what treatments may be effective

## 3- Test Treatments on Models

- Screen existing drugs to determine if they can be repurposed
- Identify therapies like ASOs, gene therapy, RNA splicing.

## 4- Safety Studies

- Conduct safety studies on potential therapies in animal models

## 5- Clinical Trials

- Conduct clinical trials in people with PPP3CA to determine if potential therapies work
- Submit for FDA and other regulatory agency approvals