

A nighttime photograph of the Dallas skyline. In the foreground, a multi-lane highway bridge is illuminated by streetlights, with light trails from traffic. The city skyline in the background features several prominent skyscrapers, including the Reunion Tower with its large illuminated sphere on the left. The text "ION283 Clinical Trial in Dallas" is overlaid in white on the cityscape.

# ION283 Clinical Trial in Dallas



# Outline

Expertise

Clinical Trial

Location

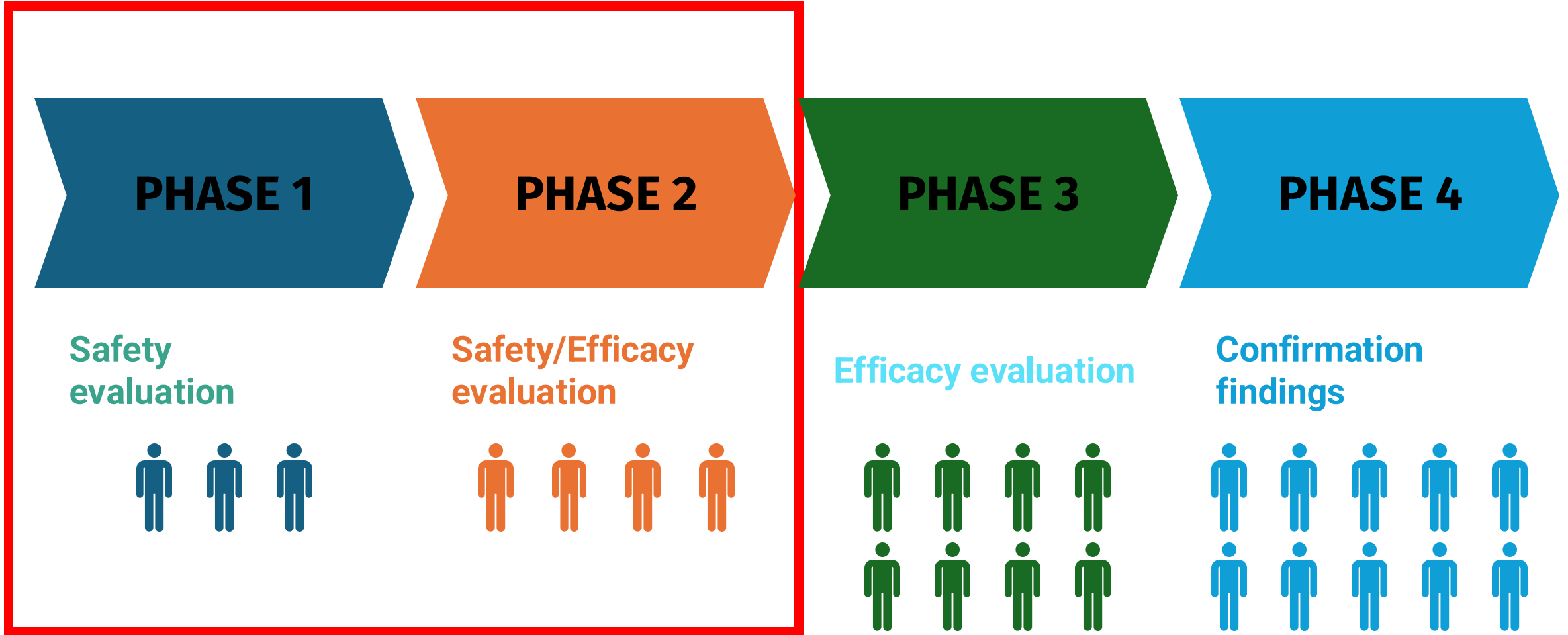
How to Prepare

# What is a clinical trial

**Clinical trials are research studies performed in people that are aimed at evaluating a new drug, device, or behavioral intervention.**



# Clinical Trial Stages



# ION 283 Regulatory process

FDA  
approval

IRB  
approval

Hospital  
Approval



# The Site Research Team

- Investigators/Doctors
- Research Coordinators
- Research Nurses
- Health care workers



# Investigators



**PI-**

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**Co-I**

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Fellow of Pediatrics  
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# Expertise and strengths

- Experienced Pediatric Neurologists
- Experience in ASO and Gene Therapy
- Experience with Epileptic encephalopathies and Lysosomal storage diseases
- Specialized Core services -MRI, PICU
- Team with experience in FDA regulated trials and regulatory compliance
- Centralized Location (DFW/Lovefield airport, nearby facilities)





Childrens Medical Center

# Proposed Phase 1/2 study

## *Primary Objective:*

To study if ION283 is safe for patients with Lafora Disease.

## *Secondary Objectives:*

To study if ION283 helps treat Lafora Disease.

## *Exploratory Objectives:*

To study biomarkers in patients with Lafora Disease before and after treatment

# ION283- Phase 1/2 Study Design



# Study Design

A Phase 1/2 Open Label Study to Evaluate the Safety and Efficacy of Intrathecally Administered ION283 in Patients with Lafora disease

A **single group** will be studied:

**N = 10**

15mg  
ION283 in  
12-week  
intervals

9 IT  
infusions

15mL  
infusion  
volume

The study consists of **2 periods**:

Screening Period: 2  
weeks

Treatment Period:  
104 weeks (24months)

The first **two participants** will be dosed first



Review of safety data

28 days after dosing



# Inclusion Criteria

- Aged 10-18 years old at the time of informed consent.
- Genetically confirmed diagnosis of Lafora disease (*EPM2A*/laforin, *EPM2B*/*NHLRC1*/malin)
- Must have LDPS score  $\geq 9$  and LDPS motor subscore of  $\geq 2$  (independent ambulation- walking 10 steps independently)

# Exclusion Criteria

- Clinically significant abnormalities in medical history
- Active Infection
- Abnormal Labs
- Problems with doing a lumbar puncture.
- Liver and Kidney problems
- Previous treatment with an Antisense Oligonucleotide
- Any investigational agent or device within the 30 days

# Recruitment



Clinicaltrials.gov



Clinical referrals



International patients



Family Foundations

# Safety Monitoring

Side Effects

Neurological  
Examinations

CSF Laboratory  
Tests

Physical  
Examinations  
and Weight

Vital Signs (HR,  
BP, respiratory  
rate, pulse)

ECGs

Blood  
Laboratory  
Tests



# Efficacy Monitoring

Annual MRI

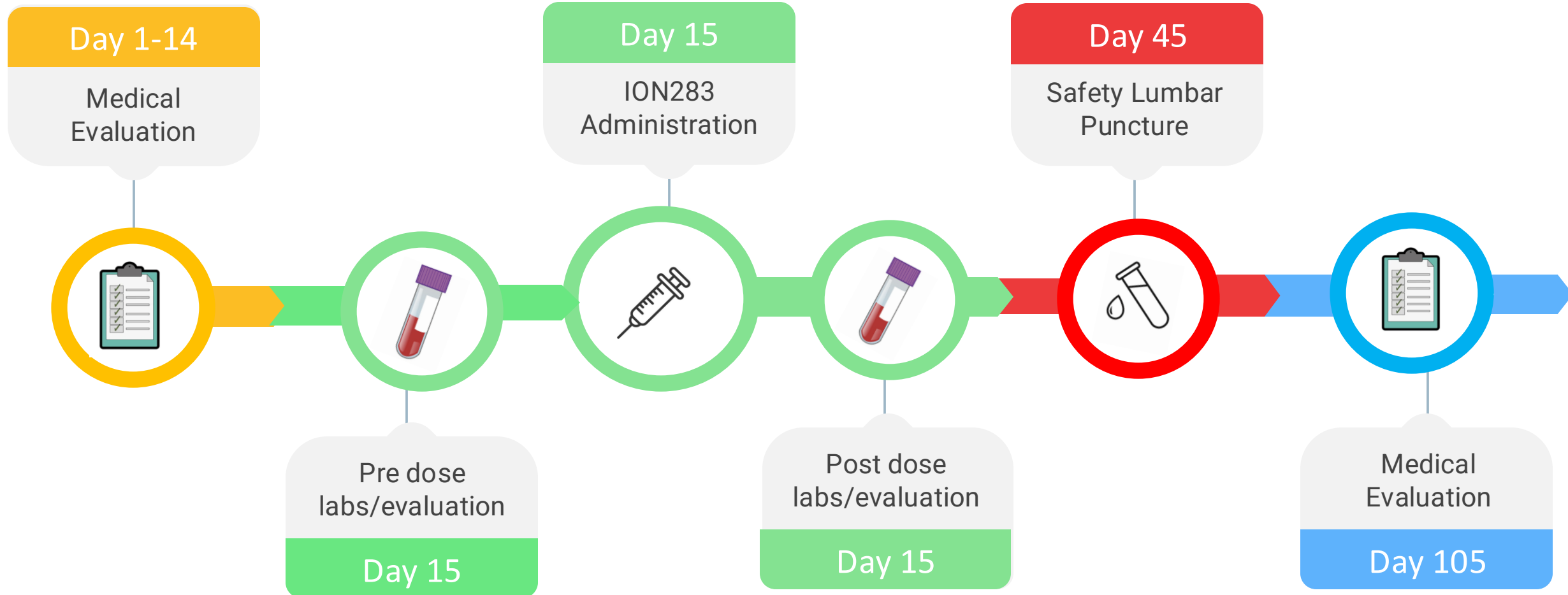
Questionnaires

Physical/  
Neurological  
Examinations

Annual 24 hr  
EEG

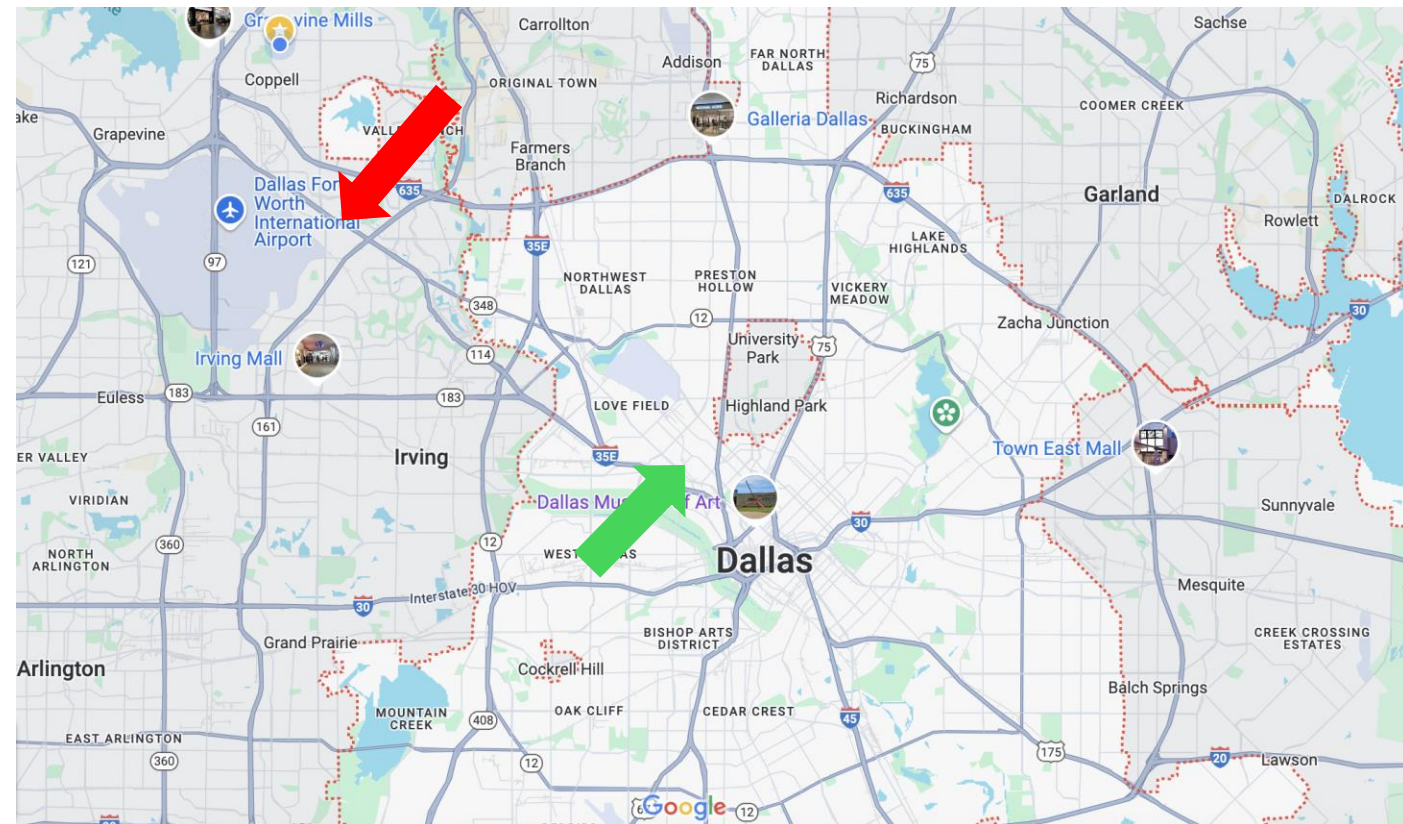
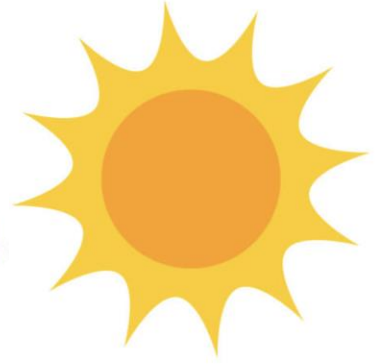
Blood  
Laboratory  
Tests

# Schedule

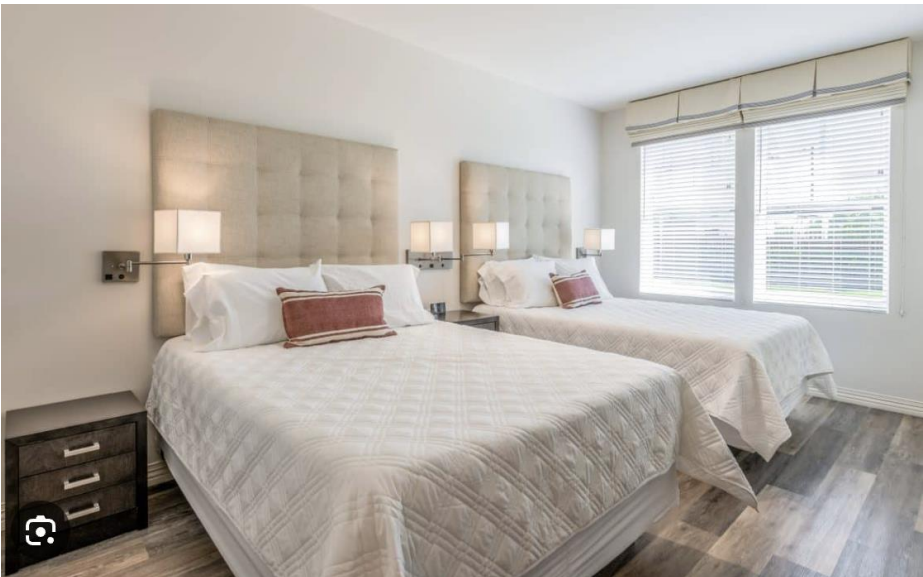
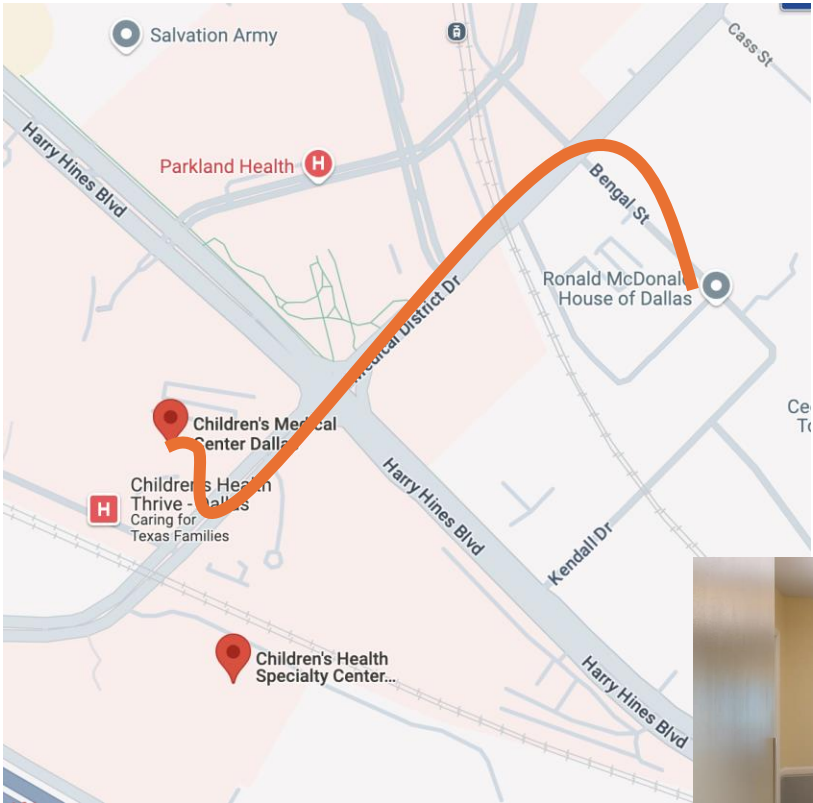


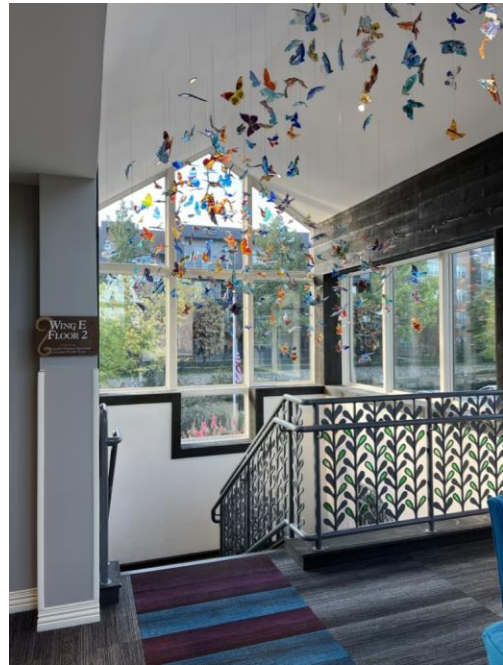
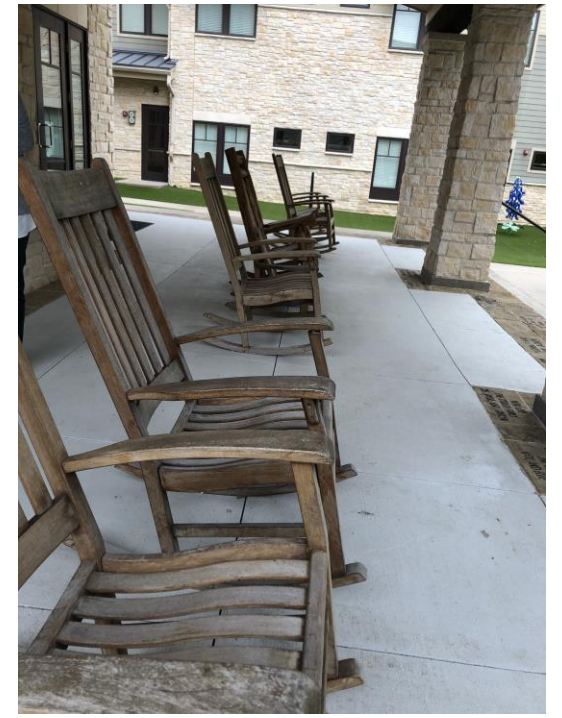
# Living in Dallas

- Limited public transport
- Diverse community
- Weather is HOT in summer!!
- Hospital location is near airport/downtown



# Ronald Macdonald House





# How to prepare

- Passports valid for more than 6 months
- Visa requirements
  - ESTE- 90 days valid for 2 years
  - B1/B2-6 months valid for up to 10 years
- Re arranging work commitments
- Childcare for siblings
- Mobile Phones
- Visa/Mastercard



# Travel/ Medical Insurance

- Travel/Medical Insurance, examples
  - [Imgglobal.com](#) - \$200 for 90 days cover up to \$100K Medical
  - [Visitorcoverage.com](#) -\$100 for 90 days cover up to \$50K Medical
  - Covers new health issues not related to any pre-existing conditions
  - Covers - Urgent care, Drs appointment, Emergency, Hospitalization, Prescription, Dental

**Scenario 1-** 15-year-old falls in the park and has injured their leg and need medical care

**Scenario 2 –** 12-year-old get an ear infection and needs antibiotics

**Scenario 3-** 18-year-old gets COVID and need cough medicine

# What to bring to Dallas

- Your child's medication
- Your own medication
- Special equipment they need
- Do not bring food! Snacks are ok
- Blanket for hospital/comfort clothing
- More details later....





**THANK YOU**

**QUESTIONS?**