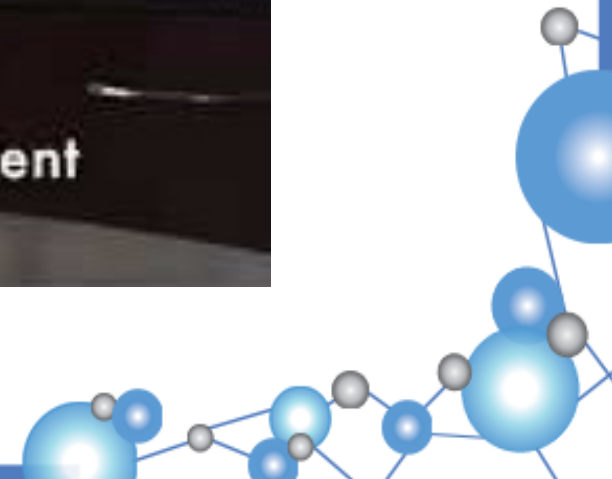




# The HI Global Registry

2020 Overview and Annual Report Highlights

# HI Global Registry Video



# What is the HI Global Registry?



# Why should I join?

YOU

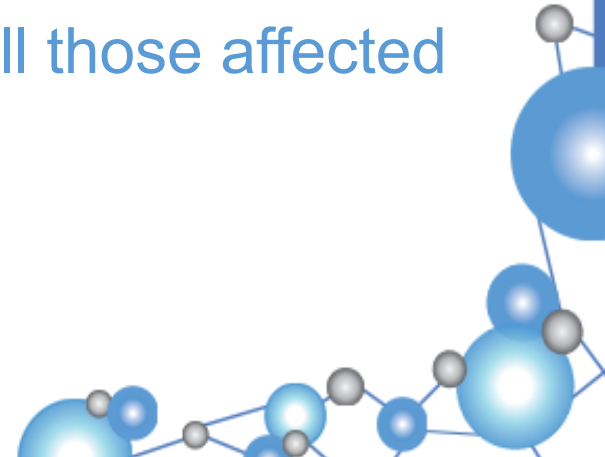


HAVE THE POWER!

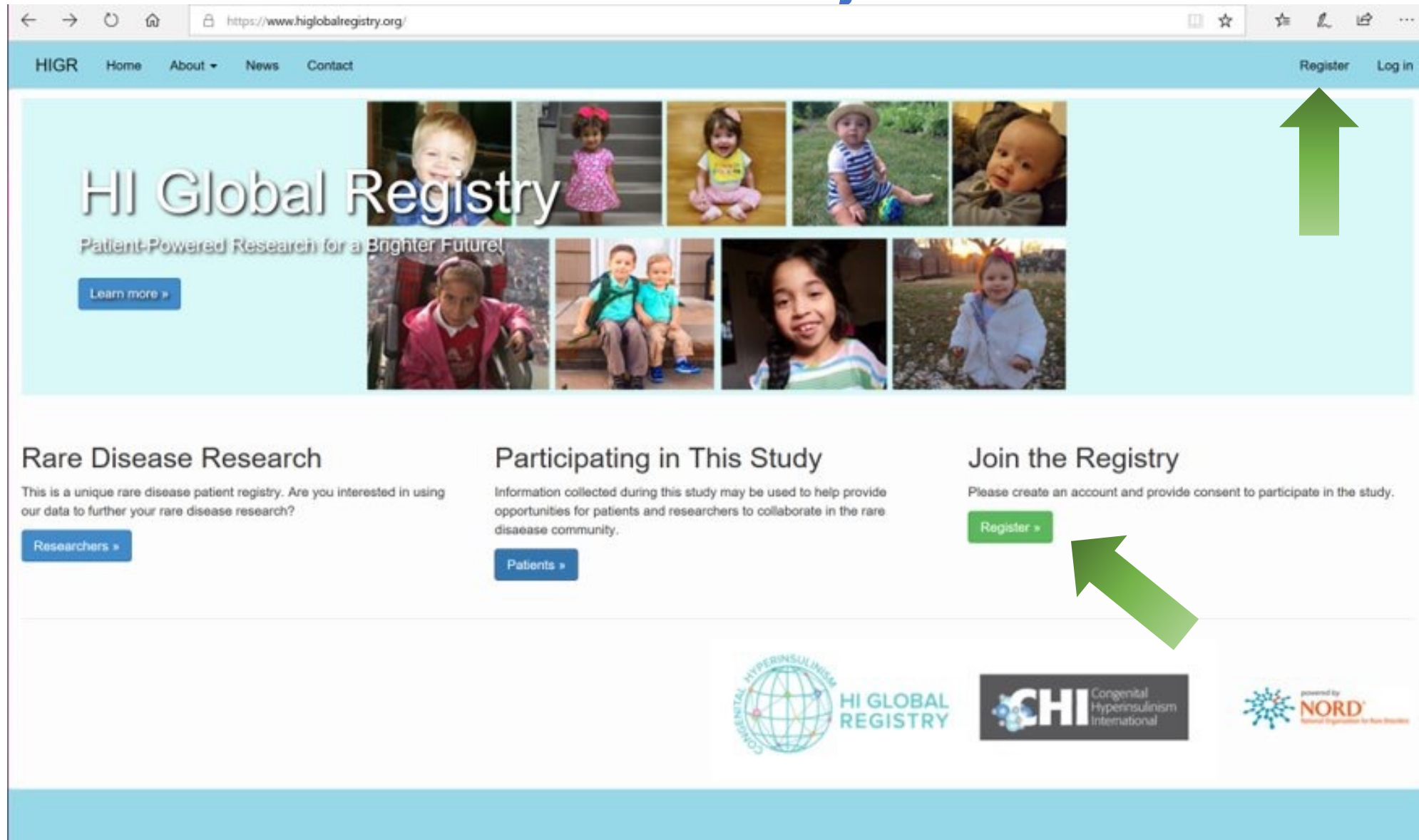
HI Global Registry  
higlobalregistry.org

*Patient-powered research  
for a brighter future*

- Generate new insights into HI
- Drive new research for treatments and cures
- Support the success of HI clinical trials
- Study and guide standards of care
- Examine factors that influence quality of life
- Improve the lives of all those affected by HI



# How do I join?



The screenshot shows the HI Global Registry website. The top navigation bar includes links for Home, About, News, and Contact, along with 'Register' and 'Log in' on the right. A large banner features the text 'HI Global Registry' and 'Patient-Powered Research for a Brighter Future!' with a 'Learn more' button. Below the banner are three main sections: 'Rare Disease Research' with a 'Researchers' button, 'Participating in This Study' with a 'Patients' button, and 'Join the Registry' with a 'Register' button. The 'Join the Registry' section includes the text 'Please create an account and provide consent to participate in the study.' The footer contains logos for the HI Global Registry, CHI (Congenital Hyperinsulinism International), and NORD (National Organization for Rare Disorders).

HI Global Registry

Patient-Powered Research for a Brighter Future!

Learn more »

Rare Disease Research

This is a unique rare disease patient registry. Are you interested in using our data to further your rare disease research?

Researchers »

Participating in This Study

Information collected during this study may be used to help provide opportunities for patients and researchers to collaborate in the rare disease community.

Patients »

Join the Registry

Please create an account and provide consent to participate in the study.

Register »

HI GLOBAL REGISTRY

CHI Congenital Hyperinsulinism International

powered by NORD National Organization for Rare Disorders



# Surveys

## Submit once

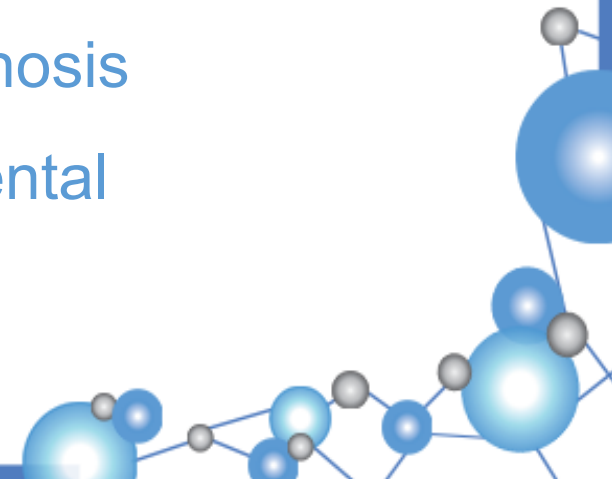
- Pregnancy
- Birth

## Longitudinal

- Glucose monitoring - 6 months
- Quality of life (Parent/ LAR) - Annual
- Quality of life (Participant) - Annual
- 

## Updatable

- Contact information
- Demographics
- Diagnosis
- Medication management
- Diet & feeding management
- Surgical management
- Other diagnosis
- Developmental



# 2020 Annual Report

congenitalhi.org/higlobalregistry/

**CHI** Congenital Hyperinsulinism International  
AWARENESS A CALL TO ACTION CARE ABOUT RARE DONATE

HOME ABOUT CHI CONGENITAL HYPERINSULINISM HI GLOBAL REGISTRY AWARENESS EVENTS DONATE RESOURCES CONTACT

f t y in i Join Our Mailing List

## The HI Global Registry

HI Global Registry 2020 Annual Report Now Available



higlobalregistry.org | Patient-powered research for a brighter future

HI Global Registry Vi...

Go to: [The HI Global Registry](#)

REGISTRY-RELATED PAGES:  
[The HI Global Registry](#)  
[HI Global Registry Fact Sheet](#)  
[HI Global Registry Q&A](#)



## 2020 Annual Report

Davelyn Eaves Hood<sup>1</sup>, Tai Pasquini<sup>2</sup>, Lauren Quinlan<sup>3</sup>, Abigail Linsmeier<sup>3</sup>, Sunny Chapel<sup>3</sup>, Isabel Calderón<sup>4</sup>, Julie Raskin<sup>5</sup>

<sup>1</sup>CHI Board President & HIGR Principal Investigator, <sup>2</sup>CHI Policy & Research Director, <sup>3</sup>A2PG, <sup>4</sup>CHI Board Emeritus and HIGR Investigator, <sup>5</sup>CHI Executive Director and HIGR Investigator

### Address for correspondence

HI Global Registry Investigators  
c/o Congenital Hyperinsulinism International  
PO Box 135  
Glen Ridge, NJ 07028, USA  
[info@higlobalregistry.org](mailto:info@higlobalregistry.org)

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# Registry Participation, by the Numbers

*HIGR, as of 2/1/2020*

Surveys submitted	Participants
Contact	177
Demographics	160
Pregnancy	164
Birth	144
Diagnosis	152
Diet & Feeding	118
Medication	115
Surgery	116
Other Diagnoses	115
Glucose Monitoring	134
Development	113
QOL Parent	129
QOL Participant	10

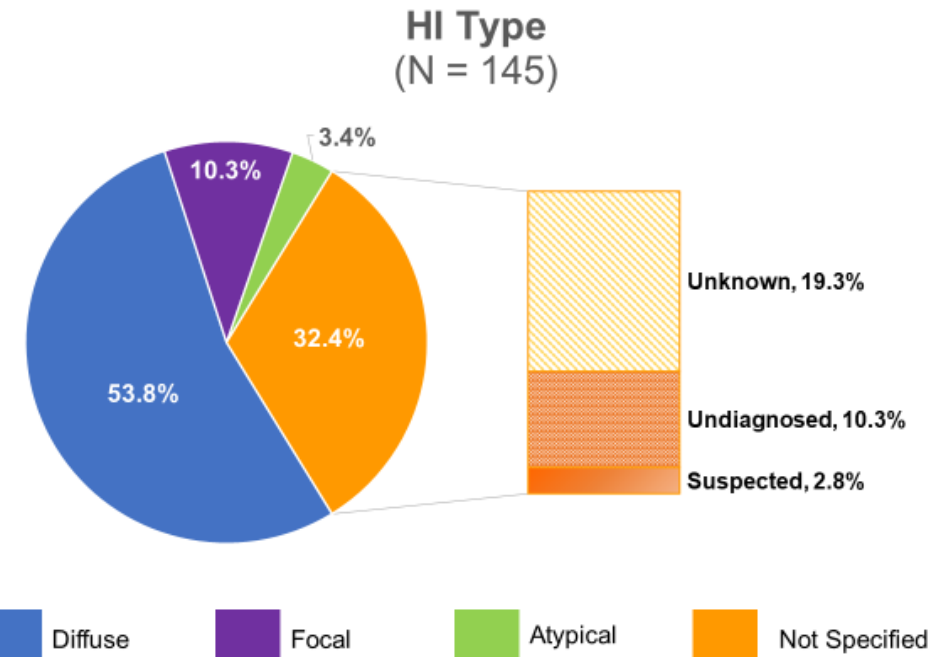
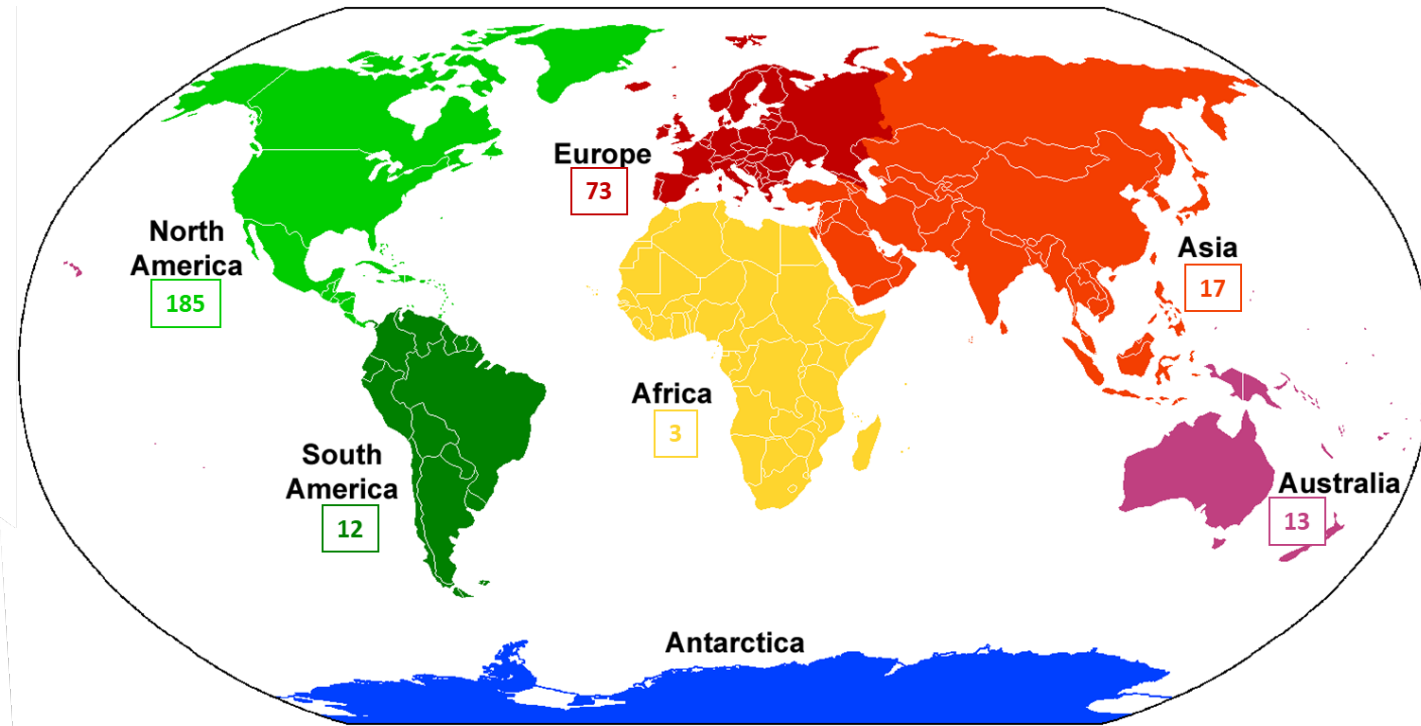
303 Participants Added  
263 Consented  
191 Begun Taking Surveys  
85 Completed Full Survey Set  
44 Countries Represented

Age	Participants
0-2 years	92
3-5 years	95
6-9 years	39
10-12 years	16
13-17 years	18
18+ years	43
Youngest	5 weeks
Oldest	58 years



# HI Prevalence Data

HIGR, as of 1/20/2020



# Medication Management

HIGR, as of 1/20/2020

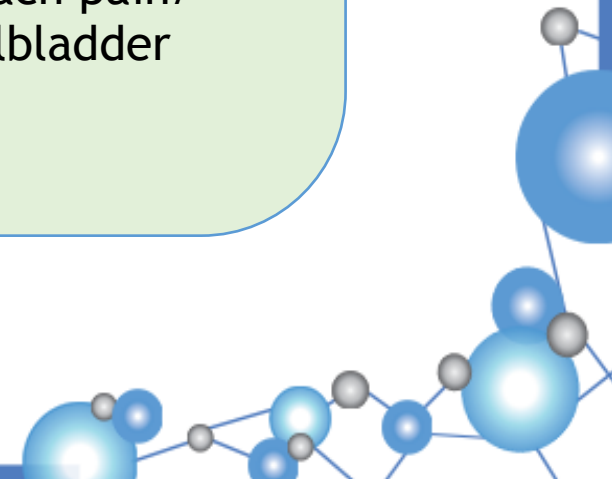
Medication use	# Currently Taking	# Total
Diazoxide	62	96
Octreotide	9	32
Sandostatin LAR	1	4
Lanreotide	10	14
Sirolimus	0	2
Steroids	1	5
Glucagon (Routine use)	2	21

*Total = Participants who indicated current and/or past use*

## Side Effects

**Diazoxide:** Increased body hair, Loss of appetite, **Continued hypoglycemia**, Swelling, Facial changes

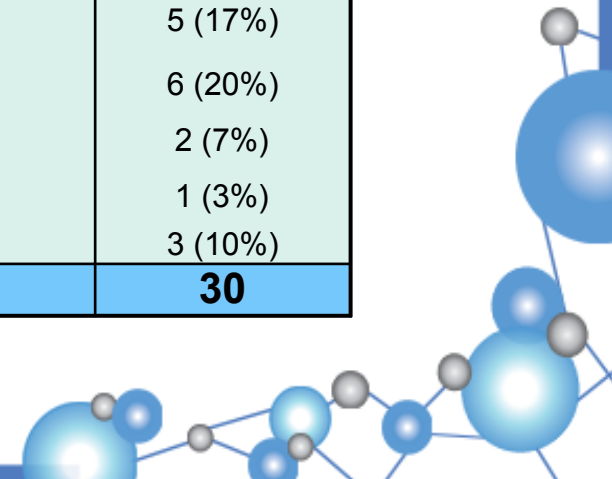
**Octreotide:** **Continued hypoglycemia**, Changes in stool, Hyperglycemia, Stomach pain/upset, Gallstones/gallbladder sludge.



# Diet & Feeding Management

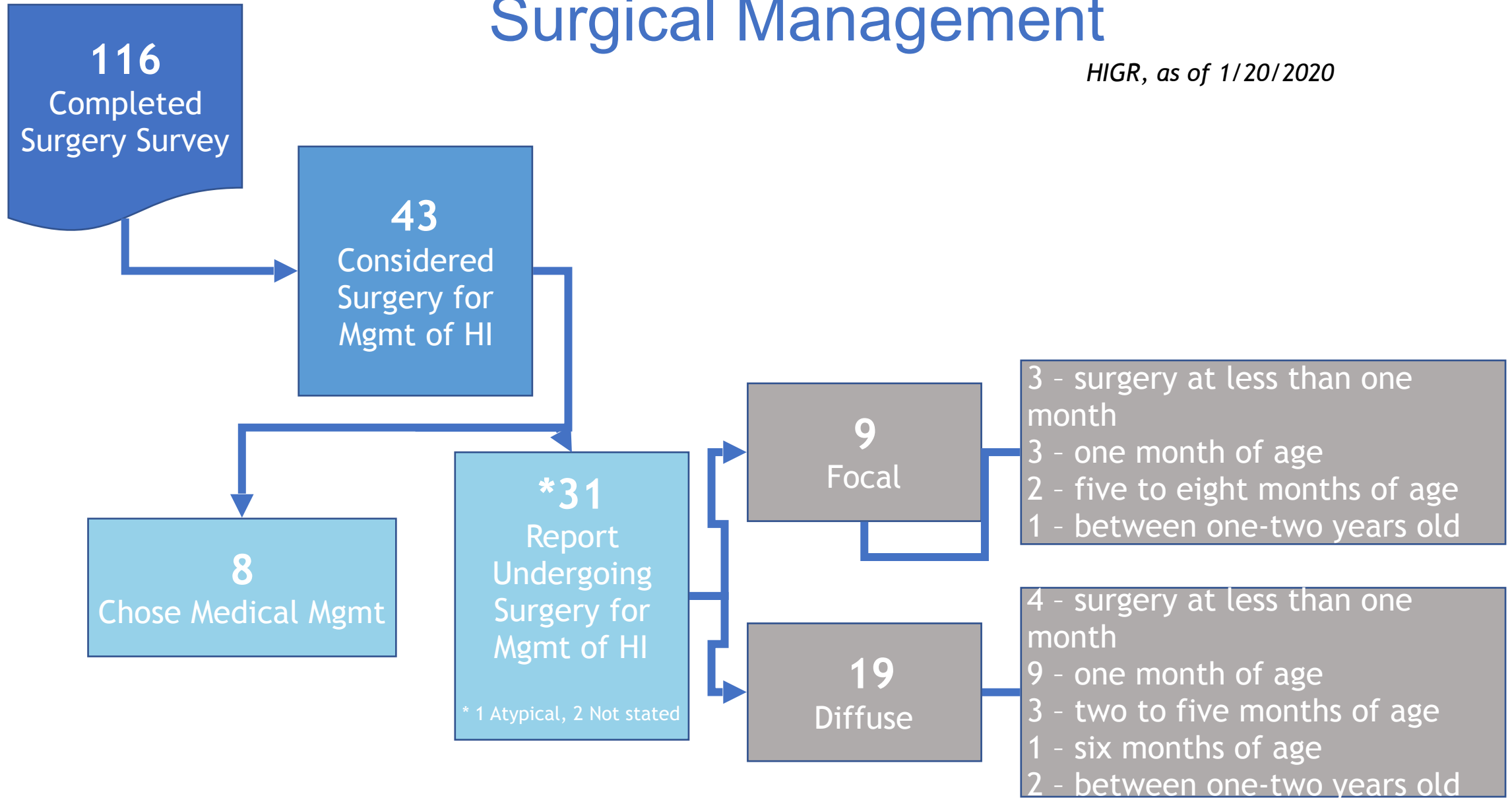
HIGR, as of 1/20/2020

Has the participant experienced any feeding issues on a regular basis (check all that apply)?	All Participants	Diffuse			Focal			Other HI Type
	W/WO Surgery	No Surgery	Pancrea-tectomy	Total	No Surgery	Pancrea-tectomy	Total	No Reported Surgery
	N (%)	N	N	N (%)	N	N	N	N (%)
No feeding issues	31 (32%)	15	2	17 (29%)	0	2	2	12 (40%)
Feeding Issues(s)	67 (68%)	29	14	43 (72%)	1	5	6	18 (60%)
<i>Poor appetite</i>	41 (42%)	17	9	26 (44%)	1	2	3	12 (40%)
<i>Refusing to eat</i>	41 (42%)	17	6	23 (39%)	1	4	5	13 (43%)
<i>Reflux</i>	33 (34%)	18	7	25 (42%)	0	2	2	6 (20%)
<i>Problems with texture</i>	28 (29%)	12	8	20 (34%)	1	2	3	5 (17%)
<i>Gagging</i>	28 (29%)	15	6	21 (36%)	0	1	1	6 (20%)
<i>Vomiting</i>	27 (28%)	13	5	18 (31%)	1	2	3	6 (20%)
<i>Uncoordinated oral skills</i>	22 (22%)	8	7	15 (25%)	1	1	2	5 (17%)
<i>Slow eating</i>	27 (28%)	14	7	21 (36%)	0	0	0	6 (20%)
<i>Coughing</i>	15 (15%)	7	6	13 (22%)	0	0	0	2 (7%)
<i>Overeating</i>	11 (11%)	7	2	9 (15%)	0	1	1	1 (3%)
<i>Other</i>	3 (3%)	0	0	0	0	0	0	3 (10%)
<b>Total</b>	<b>98</b>	<b>44</b>	<b>16</b>	<b>60</b>	<b>1</b>	<b>7</b>	<b>8</b>	<b>30</b>



# Surgical Management

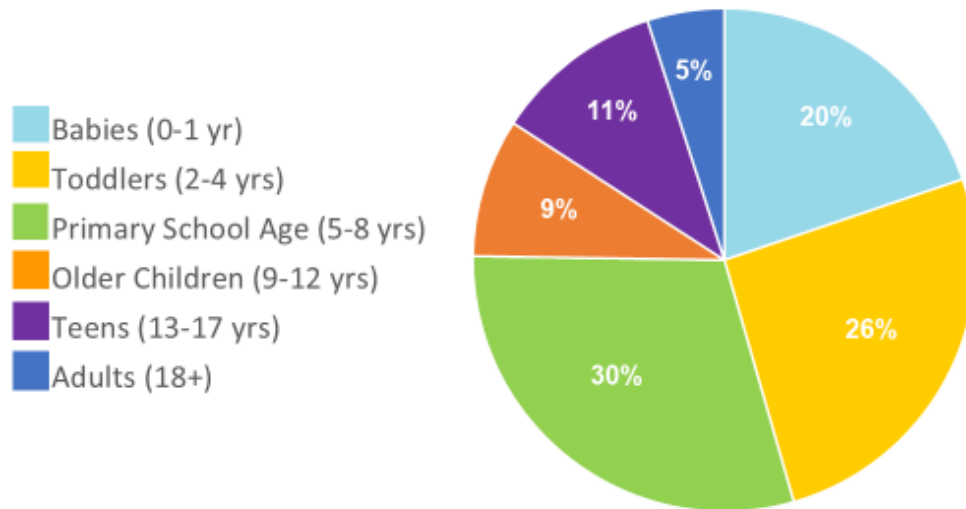
*HIGR, as of 1/20/2020*



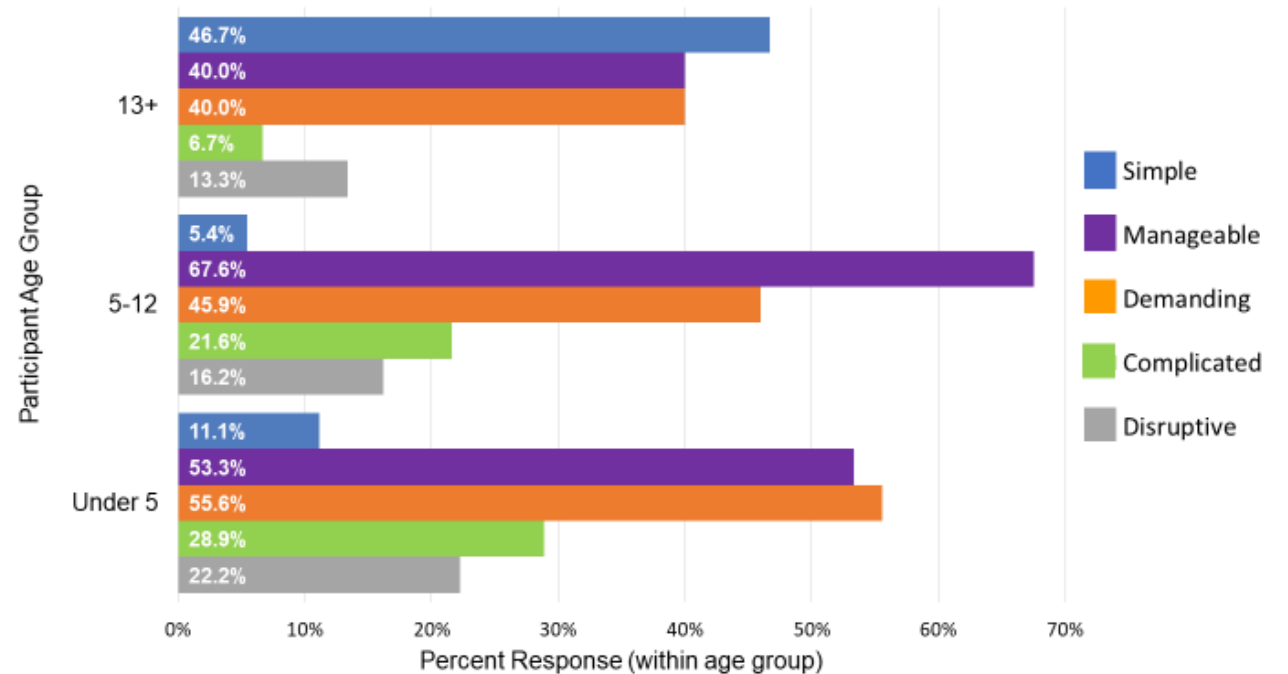
# Parent Quality of Life (QOL)

HIGR, as of 2/1/2020

Age Group of Participants Represented  
in Parent QOL Survey (N = 101)



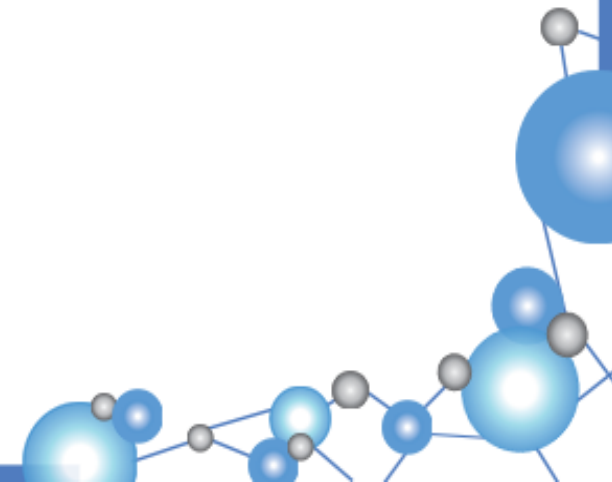
Parent HI Management Response, by Age Group  
(Under 5yrs, N = 45) (5-12 yrs, N = 37) (13+ yrs, N = 15)





# Call to Action

- Register.
- Confirm your contact preferences.
- Consent to participate in the surveys.
- Set aside the time to complete ALL surveys.
- Update survey questions.
- Complete longitudinal surveys.



# Patient-Driven Research

## What's Next for HIGR?

- Beginning to fill the gap in published HI literature
- Informing the work of CZI Rare As One Collaborative Research Network
- Supporting clinical trials for new drug development
- Piloting the Rare Disease Cures Accelerator initiative with C-PATH



Considering patients' perspectives makes it more likely that researchers will ask the right questions, conduct the studies best designed to answer them, and produce results more relevant to everyday care and more likely to be adopted in practice.

-- *Health Affairs* (10.1377/hblog20160519.054923)

