

cHI Quantitative Market Landscape Marketing Survey

Survey Draft Version: 1/5/26

Project Specifications & Sample Plan

- 30-minute online survey
- US-only
- Exhaust target list first then supplement with panel

Specialty	Quota
Ped Endo	40
Endo	10
Total	50

Master Programming Directions

- For all NUMERIC questions with multiple boxes, do not force ZERO entry in each box and auto-punch ZERO where appropriate
- For all NUMERIC questions with row/column specific masking logic, auto-punch masked cells with ZERO
- For all OE questions, do not limit number of characters allowed

Survey Outline

- Section S: Screening Criteria
- Section A: Current cHI Population & Treatment
- Section B: Client Product cHI Awareness, Trial and Use
- Section C: Client Product cHI Promotion
- Section D: cHI Treatment Perceptions
- Section E: cHI Patient Records
- Section F: CLIENT PRODUCT TPP Evaluation & Future Prescribing
- Section G: Physician cHI Attitudes and Demographics

Survey Formatting

- **GRAY TEXT** is for internal and programming use only and will not be displayed to respondents
- **BLUE TEXT** denotes question number and question purpose
- **RED TEXT**, if present, denotes input or decision is needed

Section S: Screening Criteria

Thank you for interest in participating in this survey which is conducted by APEX Market Research Services LLC, an independent market research agency, on behalf of a pharmaceutical company.

This market research is aimed to gain your views and is not intended to be promotional. Participation is voluntary and you can refuse to answer questions or withdraw from the interview/survey at any time. APEX Market Research Services LLC complies with all laws that protect your personal information (e.g. GDPR) and the following industry and market research codes of conduct: EphMRA (European Pharmaceutical Market Research Association), ESOMAR (European Society for Opinion and Marketing Research) and MRS (Market Research Society) .

We would prefer not to reveal the name of the pharmaceutical company until the end of the interview, just in case knowing this biases any responses.

Any information you provide us with will be treated as confidential, it will be combined with feedback from others like yourself. You will remain anonymous.

[NEW SCREEN]

The purpose of the research is to increase understanding of your management of congenital hyperinsulinism (cHI).

Upon completion, we will provide you with an honoraria for your time as indicated in the invitation you received to participate.

AE1 AE Disclosure

AE1	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: 3
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We are asked to pass on to our client details of adverse events and product quality complaints that are mentioned during market research interviews/surveys. Although this is a market research interview/survey and what you say will, of course, be treated in confidence, should you raise during the discussion/survey an adverse event in a specific patient, we will need to report this even if it has already been reported by you directly to the company or the regulatory authorities. In such a situation, you will be asked whether you are willing to waive the confidentiality given to you under the Market Research Codes of conduct, specifically in relation to that adverse event. Everything else you say during the course of the interview/survey will continue to remain confidential.

Are you happy to proceed with the interview on this basis?

(Please select one option.)

- 1 **YES** – Willing to be contacted by the client safety team for more information about the AE
- 3 **NO – Unwilling to be contacted by the client safety team but still willing to participate**
- 2 **NO – I am not willing to proceed** and wish to end the interview/survey here

S10 Medical Profession

S10	INCLUDE: ALL	LOGIC: SINGLE SELECTION; RANDOMIZE ANCHORING OTHER	TERMINATE: 2 OR 3 OR 99
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What is your medical profession?
(Please select one option)

- 1 Physician
- 2 Nurse Practitioner
- 3 Physician Assistant
- 99 Other (Please specify _____)

S20 Medical Specialty

S20	INCLUDE: ALL	LOGIC: SINGLE SELECTION; RANDOMIZE ANCHORING OTHER	TERMINATE: 1,3,4,5,6 OR 99
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Which of the following best describes your primary medical specialty?
(Please select one option)

- 1 Gastroenterology
- 2 Endocrinology
- 3 Primary care
- 4 Neurology
- 5 Rheumatology
- 6 Internal medicine
- 7 Pediatric endocrinology
- 99 Other (Please specify _____)

S30 Board Certification

S30	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: 3
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Are you board certified or board eligible in your specialty?
(Please select one option)

- 1 Board certified
- 2 Board eligible
- 3 Neither

S40 US State

S40	INCLUDE: ALL	LOGIC: DROP DOWN MENU, 50 STATES + DC AND PR	TERMINATE: VT, DC
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In which state do you primarily practice?
(Please select one option)

[DD MENU 50 STATES + DC]

S50 Time in Direct Patient Care

S50	INCLUDE: ALL	LOGIC: NUMERIC 0-100, TOTAL MUST SUM TO 100; DO NOT RANDOMIZE	TERMINATE: S50_1 < 50
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On average, what percent of your professional time is spent on each of the following?
(Please fill in boxes, whole numbers only. Please note your total should sum to 100%.)

- | | | | |
|---|--|--------------|--------------------------------------|
| 1 | | _____ % | Direct patient care |
| 2 | | _____ % | Teaching / academia |
| 3 | | _____ % | Research / clinical trials |
| 4 | | _____ % | Administration and coordinating care |
| 5 | | _____ % | Other (Please specify _____) |
| | | SHOW
SUM% | Total |

S60 Years in Practice

S60	INCLUDE: ALL	LOGIC: NUMERIC 1-99	TERMINATE: <2 or >35
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How long have you personally been in practice since your endocrinology fellowship?
(Please fill in boxes, whole numbers only.)

_____ years in practice

S70 Number of Patients of Each Type

S70	INCLUDE: ALL	LOGIC: NUMERIC 0-9999; RANDOMIZE ROWS	TERMINATE: S70_5=0
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In a **typical one-month period**, how many patients do you personally treat for each of the following conditions? Please exclude patients for whom you are only consulting for diagnostic purposes.

(Please fill in boxes, whole numbers only.)

- | | | Number of patients in a typical month | |
|---|--|---------------------------------------|--|
| 1 | | _____ | Type 1 diabetes (T1D) |
| 2 | | _____ | Type 2 diabetes (T2D) |
| 3 | | _____ | Obesity |
| 4 | | _____ | Genetic endocrine disorders (Turner syndrome, Prader-Willi syndrome) |
| 5 | | _____ | Congenital hyperinsulinism (cHI) |
| 6 | | _____ | Growth and puberty disorders (short stature) |
| 7 | | _____ | Pituitary gland disorders (diabetes insipidus, hypopituitarism) |

S80 cHI Patients by Focal/Diffuse

S80	INCLUDE: ALL	LOGIC: NUMERIC 0-9999; SUM=S70_5	TERMINATE: S80_2=0
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How many of the [INSERT S70_5] **congenital hyperinsulinism (cHI) patients** that you treat fall into the following disease categories?

Please fill in boxes, whole numbers only.)

Number of cHI patients categorized as:		
1	_____	Focal cHI - only a localized region or "focal lesion" of beta cells in the pancreas over-secretes insulin
2	_____	Diffuse cHI - all of the insulin-producing beta cells in the pancreas overproduce insulin
CALC SUM		

S85 Patient Record Agreement

S85	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: 2
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Sections within this survey will require you to generate individual **congenital hyperinsulinism (cHI)** patient information like:

- Patient height
- Patient weight
- Patient diagnosis/treatment history
- Patient comorbidities
- Current patient medication dosing
- Patient functioning

All of this information will remain anonymous. Will you be able to generate information as you complete the survey?

(Please select one option)

- | | |
|---|---------------------------|
| 1 | <input type="radio"/> Yes |
| 2 | <input type="radio"/> No |

S90 Government Employee

S90	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: NONE
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SHOW IF US: Are you a full-time, part-time or contract employee within the Federal, State, or Local government? (Please select one option)

- | | |
|---|---------------------------|
| 1 | <input type="radio"/> Yes |
| 2 | <input type="radio"/> No |

S100 Government Employee Market Research

S100	INCLUDE: S90=1	LOGIC: SINGLE SELECTION	TERMINATE: 2
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As a government employee, are you able to participate in marketing research and receive fair market compensation for it?

(Please select one option)

- 1 | Yes
- 2 | No

S110 P&T Committee Member

S110	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: NONE
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Are you a member of the P&T Committee of a health plan, pharmacy benefit manager, or other healthcare provider or payer?

(Please select one option)

- 1 | Yes
- 2 | No

S120 P&T Committee Member Market Research

S120	INCLUDE: S110=1	LOGIC: SINGLE SELECTION	TERMINATE: 2
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Does your participation in this marketing research study and acceptance of honoraria comply with applicable ethics rules of the P&T Committee, including standards for conflict of interest, disclosure, and recusal?

(Please select one option)

- 1 | Yes
- 2 | No

S130 Conflicts of Interest

S130	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: 1
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Do you work full-time, work part-time, or provide consultative services (e.g., as clinical investigator, researcher, consultant, or adviser) for any of the following?

- Advertising agency
- Marketing research firm
- Marketing or healthcare consulting firm
- Manufacturer of healthcare products, including pharmaceuticals, diagnostics or medical equipment
- Distributor of healthcare products, including pharmaceuticals, diagnostics or medical equipment
- Food & Drug Administration
- Kaiser Permanente

(Please select one option)

- 1 | Yes
2 | No

S140 MR Participation

S140	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: 1
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Have you participated in any other market research regarding congenital hyperinsulinism (cHI) in the past 3 months?

(Please select one option)

- 1 | Yes
2 | No

GDPR1 NON-EUROPE

GDPR1	INCLUDE: ALL	LOGIC: SINGLE SELECTION	TERMINATE: 2
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For the purposes of this research, your personal data, including your name, phone number and email address may be shared with the market researchers conducting this study who are located in the United States. Your personal data will never be shared with the end client sponsoring this research. Any personal data collected about you is used for market and opinion research purposes only and there are security safeguards in place to prevent risks such as loss, misuse, unauthorized access or unauthorized use. Your personal information will be retained for as long as necessary to complete this research and then destroyed. You have rights to confidentiality, anonymity, and can withdraw at any time. You also have the right to access your personal information and/or request that it be erased at any time.

To learn more about the Data Privacy Framework program, and to view our certification page, please visit www.dataprivacyframework.gov.

Do you agree to provide your personal data and consent that it be processed as stated above?
(Please select one option)

- 1 Yes
- 2 No

Introduction

Congratulations, you have qualified to complete this survey regarding the treatment of **diffuse congenital hyperinsulinism (cHI)**.

Please complete this survey based on your **individual prescribing practices** for the **diffuse congenital hyperinsulinism (cHI) patients you personally treat and/or manage**.

Please also be candid in your responses. The information collected in this survey will be used for market research purposes only. Completion of this survey should take approximately 30 minutes or less.

We sincerely appreciate your time and your valuable input.

Please click "Continue" to begin the survey.

Section A: Current cHI Population & Treatment

A10 Diffuse cHI Patients by Age

A10	INCLUDE: ALL	LOGIC: NUMERIC 0-9999; SUM=S80_2
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How many of the [INSERT S80_2] **diffuse congenital hyperinsulinism (cHI)** patients that you treat fall into the following age ranges?

Please fill in boxes, whole numbers only.)

Number of diffuse cHI patients in each age category:		
1	_____	<1 year old
2	_____	1-3 years old
3	_____	4-6 years old
4	_____	7-18 years old
5	_____	>18 years old
CALC SUM		

A15 Status of Current cHI Patients

A15	INCLUDE: ALL	LOGIC: NUMERIC 0-9999; SUM=S80_2
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How many of the [INSERT S80_2] **diffuse congenital hyperinsulinism (cHI)** patients that you treat fall into the following categories?

Number of diffuse cHI patients in each category:		
1	_____	Are adequately managed on their current therapy(s)
2	_____	Have recently switched therapy(s) and are in the evaluation stage
3	_____	Are achieving some success on current therapy(s) but results are not optimal
4	_____	Are not doing well and are candidate(s) for therapy switching if one was available
CALC SUM		

A20 Treatment Drivers (Max-Diff)

A20

INCLUDE:
ALL

LOGIC:
REPEAT QUESTION FOLLOWING MAX-DIFF DESIGN

Which of the following attributes for **diffuse congenital hyperinsulinism (CHI)** treatment decision-making is least important and most important to you?

You will be evaluating X sets of attributes. For each set of five attributes that you see, please select the box next to the attribute that is least important and the attribute that is most important to you.

Least Important		Most Important
<input type="radio"/>	Attribute 1	<input type="radio"/>
<input type="radio"/>	Attribute 2	<input type="radio"/>
<input type="radio"/>	Attribute 3	<input type="radio"/>
<input type="radio"/>	Attribute 4	<input type="radio"/>
<input type="radio"/>	Attribute 5	<input type="radio"/>

List of attributes to be tested

Mechanism of action

Efficacy in patients, regardless of genetics

Speed of efficacy onset

Reduction in hypoglycemia frequency

Risk of edema

Risk of excessive hair growth

Risk of tachycardia

Level of treatment emergent AEs

Risk of discontinuation due to AEs

Mode of administration (e.g. oral vs. IV)

Dosing frequency

Ease of titration

Can be used in combination therapy

Extensiveness of clinical data

Indication for patients as young as three months old

A25 Recent Approval Unaided Awareness

A25	INCLUDE: ALL	LOGIC: OPEN END
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Please list all of the therapies you are aware of as approved in the past 6 months for the treatment of **diffuse congenital hyperinsulinism (cHI)**.

(Enter each treatment in a separate box below.)

1	<input type="text"/>
2	<input type="text"/>
3	<input type="text"/>
4	<input type="text"/>
5	<input type="text"/>

99 I am not aware of or cannot recall the names of any products that were approved in the past 6 months for the treatment of **diffuse congenital hyperinsulinism (cHI)**

A30 cHI Pipeline Manufacturer Unaided Awareness

A30	INCLUDE: ALL	LOGIC: MULTI SELECT; RANDOMIZE
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Which of the following companies are you aware of as having therapies approved in the past 6 months for the treatment of **diffuse congenital hyperinsulinism (cHI)**?

(Select all that apply)

- | | | |
|----|--------------------------|-------------------|
| 1 | <input type="checkbox"/> | GSK |
| 2 | <input type="checkbox"/> | Amylyx |
| 3 | <input type="checkbox"/> | Roche |
| 4 | <input type="checkbox"/> | Hanmi |
| 5 | <input type="checkbox"/> | Biogen |
| 6 | <input type="checkbox"/> | Novartis |
| 7 | <input type="checkbox"/> | Rezolute |
| 8 | <input type="checkbox"/> | Zealand |
| 98 | <input type="radio"/> | Don't know |
| 99 | <input type="radio"/> | None of the above |

A30 Recent cHI Approval Aided Awareness

A30	INCLUDE: ALL	LOGIC: CAROUSEL SCALE; RANDOMIZE
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For each therapy shown in **blue**, please select the response that best characterizes your familiarity with it as a treatment either in development or recently approved for **diffuse congenital hyperinsulinism (cHI)**?

Once you select a response, the next therapy/mechanism of action will appear. When you have finished rating each product, click the 'Continue' button to proceed. (Select one option)

INSERT PRODUCT FROM LIST BELOW

1	2	3	4
Not at all familiar with it (Never heard of it for cHI)	Slightly familiar with it (Heard of it, but not very familiar with it)	Somewhat familiar with it (know the basics of its administration, dosing, efficacy, safety)	Very familiar with it (know the details of its administration, dosing, efficacy, safety)

ITEMS TO INCLUDE:

- 1 Avexitide
- 2 Dasiglucagon
- 3 Ersodetug
- 4 HM-15136
- 5 Insulin receptor allosteric modulator
- 6 GLP-1 receptor antagonist
- 7 Glucagon analogue

Section B: Client Product cHI Awareness, Trial and Use

B5 Recent cHI Approval Aided Awareness Details

B5	INCLUDE: SHOW IF A30_3 >2	LOGIC: OPEN END;
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For the therapy shown, what do you know or recall hearing about it as a treatment for **diffuse congenital hyperinsulinism (cHI)**?

Please enter your responses below and be as specific as possible.

A. A30 Response

B10 Sources of Recent cHI Approval Awareness

B10	INCLUDE: SHOW IF A30_3 >2	LOGIC: MULTI SELECT; RANDOMIZE
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Have you ever participated in or had exposure to any of the following educational activities for **Client Product** as a treatment for **diffuse congenital hyperinsulinism (cHI)**? (Select all that apply)

- 1 Journal articles
- 2 Met with **Client Product** sales representative (either face-to-face or virtual)
- 3 Met with **Client** Medical Science Liaison (either face-to-face or virtual)
- 4 Participated in webinar hosted by **Client**
- 5 Visited **Client Product** webpage
- 6 CME activities
- 7 Conference presentations
- 8 Visiting professor lecture in my area (live speaker forum)
- 9 Market research/surveys
- 10 Colleagues
- 11 Managed care overview/guidelines
- 12 Have prior clinical trial experience with **Client Product** in **congenital hyperinsulinism (cHI)**
- 13 My own independent research
- 98 None of these activities [EXCLUSIVE] [ANCHOR]
- 99 Other – please specify: [OE] [ANCHOR]

B13 Influence of Sources of Client Product Information

B13	INCLUDE: SHOW IF A30_3 >2	LOGIC: 0-100; ONLY SHOW OPTIONS SELECTED IN B10
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How influential were the following resources in learning about **Client Product** in your **diffuse congenital hyperinsulinism (cHI)** patients? Please allocate 100 points across the following sources of information where higher points indicates a greater influence on learning about **Client Product** and lower points indicates less of an influence on learning about **Client Product**.

If two or more sources of information are equally influential, please allocate the same number of points to them.

(Please fill in boxes, whole numbers only.)

- 1 Journal articles
- 2 Met with **Client Product** sales representative (either face-to-face or virtual)
- 3 Met with **Client** Medical Science Liaison (either face-to-face or virtual)
- 4 Participated in webinar hosted by **Client**
- 5 Visited **Client Product** webpage
- 6 CME activities
- 7 Conference presentations
- 8 Visiting professor lecture in my area (live speaker forum)
- 9 Market research/surveys
- 10 Colleagues
- 11 Managed care overview/guidelines

- 12 _____ Have prior clinical trial experience with **Client Product** in **congenital hyperinsulinism (cHI)**
- 13 _____ My own independent research
- 99 _____ Other – please specify: [OE] [ANCHOR]
- 100 _____ Total (will equal 100)

B15 Learn Something New About Client Product

B15	INCLUDE: SHOW IF A30_3 >2	LOGIC: SINGLE SELECTION
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In the PAST THREE MONTHS, have you learned any new information about **Client Product** as a treatment for **diffuse congenital hyperinsulinism (cHI)**? (*Select one*)

- 1 Learned nothing new
- 2 Learned a little more
- 3 Learned a lot more

B20 Status of Current cHI Patients

B20	INCLUDE: SHOW IF A30_3 >2	LOGIC: NUMERIC 0-999; COLUMN A ≤S80_2
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How many of the [INSERT S80_SUM] **diffuse congenital hyperinsulinism (cHI)** patients under your care have you EVER treated with **Client Product**?

RANGE PER CELL: 0-999				
		Total patients <u>currently</u> treated	Total patients <u>discontinued</u>	Total patients <u>ever</u> started
		A	B	C
1	Client Product			

B25 Timing for Potential First Client Product Patient

B25	INCLUDE: B20_COLUMN C=0	LOGIC: SINGLE SELECTION
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When do you anticipate placing your first patient on **Client Product** as a treatment for **diffuse congenital hyperinsulinism (cHI)**? (*Select one*)

- 1 Within the next month
- 2 Within the next 1 to 3 months
- 3 Within the next 4 to 6 months
- 4 Within the next 7 months to a year
- 5 More than a year
- 6 Never

B30 Prescribing of Client Product in Past 6 Months

B30	INCLUDE: B20 COLUMN C>0	LOGIC: NUMERIC 0-999; ≤B20 COLUMN C
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In the PAST SIX MONTHS, to how many patients did you prescribe **Client Product** for **diffuse congenital hyperinsulinism (cHI)** for the first time?

1	Number of Diffuse cHI patients in PAST SIX MONTHS: _____	Patients prescribed Client Product for diffuse congenital hyperinsulinism (cHI) for the first time
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B35 Barriers to use of Client Product

B35	INCLUDE: ALL	LOGIC: MULTI SELECT; RANDOMIZE
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What are the barriers to your use of **Client Product** (or increased use of **Client Product**)?

- 1 Lack of familiarity/experience
- 2 Concerns about efficacy
- 3 Concerns about safety
- 4 Concerns about tolerability
- 5 Out of pocket costs
- 6 Reimbursement hassles
- 7 No/limited advantage over other options
- 8 Patient reluctance
- 9 Lack of promotion/manufacture support
- 10 Delivery device
- 11 Lack of samples available
- 98 None of these activities [EXCLUSIVE] [ANCHOR]
- 99 Other – please specify: [OE] [ANCHOR]

B40 Primary barriers to use of Client Product

B40	INCLUDE: ALL	LOGIC: SINGLE SELECTION
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What is the PRIMARY barrier to your use of **Client Product** (or increased use of **Client Product**)? (Select one)

- 1 Lack of familiarity/experience
- 2 Concerns about efficacy
- 3 Concerns about safety
- 4 Concerns about tolerability
- 5 Out of pocket costs
- 6 Reimbursement hassles
- 7 No/limited advantage over other options
- 8 Patient reluctance
- 9 Lack of promotion/manufacture support
- 10 Delivery device
- 11 Lack of samples available
- 98 None of these activities [EXCLUSIVE] [ANCHOR]
- 99 Other – please specify: [OE] [ANCHOR]

B45 Prescribing of Client Product in Next 6 Months

B45	INCLUDE: ALL	LOGIC: NUMERIC 0-999; ≤S80_2
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In the NEXT SIX MONTHS, to how many patients do you expect to prescribe **Client Product** for **diffuse congenital hyperinsulinism (cHI)** for the first time?

1	<p>Number of Diffuse cHI patients in NEXT SIX MONTHS:</p> <p>_____</p>	<p>Patients prescribed Client Product for diffuse congenital hyperinsulinism (cHI) for the first time</p>
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B50 Prescribing of Client Product as Monotherapy or Combination Therapy

B50	INCLUDE: B20 COLUMN C>0	LOGIC: NUMERIC 0-999; ≤B20 COLUMN C
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Of your [INSERT A55 COLUMN C] patients initiated on **Client Product** for **diffuse congenital hyperinsulinism (cHI)**, how many fall in the following categories?

1	<p>Number of Diffuse cHI patients RECEIVING Client Product:</p> <p>_____</p>	<p>Client Product patients initiated as a monotherapy with no other concomitant therapies</p>
2	<p>_____</p> <p>B20c</p>	<p>Client Product patients initiated in combination with other therapies</p> <p>Total will equal [INSERT B20c]</p>

B55 Method of Accessing First Dose of Client Product

B55	INCLUDE: B20 COLUMN C>0	LOGIC: NUMERIC 0-999; ≤B20 COLUMN C
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Of your [INSERT B20 COLUMN C] patients initiated on **Client Product** for **diffuse congenital hyperinsulinism (cHI)**, how many fall in the following categories?

Number of Diffuse cHI patients RECEIVING Client Product:		
1	_____	Client Product patients initiated as part of a clinical trial
2	_____	Client Product patients initiated via secured insurance coverage
3	_____	Client Product patients initiated via patient access program (no insurance coverage)
4	_____	Client Product patients initiated via samples/free drug
5	_____	Client Product patients initiated via other [ANCHOR]
	B20c	Total will equal [INSERT B20c]

B60 Method of Accessing First Dose of Client Product

B60	INCLUDE: B55_4>0	LOGIC: NUMERIC 0-999; ≤B20 COLUMN C
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Of your [INSERT B20 COLUMN C] **diffuse congenital hyperinsulinism (cHI)** patients initiated on **Client Product** via samples/free drug, how many are continuing treatment via samples/free drug vs. continuing treatment through insurance coverage?

Number of Diffuse cHI patients RECEIVING Client Product:		
1	_____	Continuing treatment via samples/free drug
2	_____	Continuing treatment after securing insurance coverage
3	_____	None of the above
	B55_4	Total will equal [INSERT B45_4]

B65 Client Product Initiation Drivers

B65	INCLUDE: B20 COLUMN C>0	LOGIC: NUMERIC 0-999; ≤B20 COLUMN C
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What were the main drivers for starting **Client Product** in your **diffuse congenital hyperinsulinism (cHI)** patients? Please allocate 100 points across the following reasons where higher points indicates greater importance to the **Client Product** decision in **diffuse congenital hyperinsulinism (cHI)** patients.

(Please fill in boxes, whole numbers only.)

- | | | |
|---|-------|--|
| 1 | _____ | Desire to try/wanted to gain clinical experience |
| 2 | _____ | Severity of patient |

3	_____	Lack of alternative options
4	_____	Mechanism of action (MOA)
5	_____	Compelling sales representative
6	_____	Patient request
7	_____	Safety profile
8	_____	Favorable market access
9	_____	Sample on hand/free drug available
10	_____	Dosing profile
11	-----	Comparative efficacy
99	_____	Other [ANCHOR]
	100%	Total (will equal 100%)

B70 Other Drivers of Client Product Initiation

B70	INCLUDE: B65_99>0	LOGIC: OPEN END;
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You indicated that there was another driver of **Client Product** initiation in your **diffuse congenital hyperinsulinism (cHI)** patients, Please explain.

Please enter your responses below and be as specific as possible.

Section C: Client Product cHI Promotion

C10 Sales Representative Interaction

C10	INCLUDE: ALL	LOGIC: SINGLE SELECTION
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When was the last time you had an interaction with a sales representative - either virtual or face-to-face - for **Client Product** as a treatment for **diffuse congenital hyperinsulinism (cHI)**?
(Select one)

- 1 Within the past week
- 2 Within the past 1 to 4 weeks
- 3 Within the past 1 to 3 months
- 4 Within the past 4 to 6 months
- 5 More than 6 months ago
- 6 Never

C15 Sales Representative Interaction Type

C15	INCLUDE: C10=1-3	LOGIC: SINGLE SELECTION
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You mentioned the last time you had an interaction with a sales representative for **Client Product** as a treatment for **diffuse congenital hyperinsulinism (cHI)**. Was that interaction face-to-face or virtual?

(Select one)

- 1 Face-to-face
- 2 Virtual

C20 erodotug Unaided Message Recall

C20	INCLUDE: C10=1-3	LOGIC: OPEN END;
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During your most recent **Client Product** representative contact, please describe the main messages communicated.

Please enter your responses below and be as specific as possible.

C25 erodotug Aided Message Recall

C25	INCLUDE: C10=1-3	LOGIC: MULTI SELECT; RANDOMIZE
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During your most recent **Client Product** representative contact, please indicate all of the messages communicated.

(Select all that apply)

- 1 Clinical trial data, package insert review
- 2 Coverage/formulary/PA/OOP expense
- 3 Comparative efficacy

- 4 Safety/tolerability
- 5 Dosing/administration profile
- 6 Sample/patient assistance program
- 7 Ideal patient types
- 99 Other – please specify: [OE] [ANCHOR]

C30 Client Product Primary Aided Message Recall

C30	INCLUDE: C10=1-3	LOGIC: SINGLE SELECTION
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What was the PRIMARY message communicated during your most recent **Client Product** representative contact?

(Select one)

- 1 Clinical trial data, package insert review
- 2 Coverage/formulary/PA/OOP expense
- 3 Comparative efficacy
- 4 Safety/tolerability
- 5 Dosing/administration profile
- 6 Sample/patient assistance program
- 7 Ideal patient types
- 99 Other – please specify: [OE] [ANCHOR]

C33 Client Product Message Believability

C33	INCLUDE: C10=1-3;	LOGIC: NUMERIC 0-100; ONLY SHOW OPTIONS SELECTED IN C25
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How believable were the messages for starting **Client Product** in your **diffuse congenital hyperinsulinism (cHI)** patients? Please allocate 100 points across the following messages where higher points indicates a more believable message and lower points indicates a less believable message.

If two or more messages are equally believable, please allocate the same number of points to them.

(Please fill in boxes, whole numbers only.)

- | | | |
|----|-------------|--|
| 1 | _____ | Clinical trial data, package insert review |
| 2 | _____ | Coverage/formulary/PA/OOP expense |
| 3 | _____ | Comparative efficacy |
| 4 | _____ | Safety/tolerability |
| 5 | _____ | Dosing/administration profile |
| 6 | _____ | Sample/patient assistance program |
| 7 | _____ | Ideal patient types |
| 99 | _____ | Other – please specify: [OE] [ANCHOR] |
| | CALC
SUM | Total (will equal 100) |

C34 Client Product Message Motivation

C34	INCLUDE: C10=1-3;	LOGIC: NUMERIC 0-100; ONLY SHOW OPTIONS SELECTED IN C25
------------	-------------------	---

How motivating (to use) were the messages for starting **Client Product** in your **diffuse congenital hyperinsulinism (cHI)** patients? Please allocate 100 points across the following messages where higher points indicates a more motivating to use message and lower points indicates a less motivating to use message.

If two or more messages are equally believable, please allocate the same number of points to them.

(Please fill in boxes, whole numbers only.)

1	_____	Clinical trial data, package insert review
2	_____	Coverage/formulary/PA/OOP expense
3	_____	Comparative efficacy
4	_____	Safety/tolerability
5	_____	Dosing/administration profile
6	_____	Sample/patient assistance program
7	_____	Ideal patient types
99	_____	Other – please specify: [OE] [ANCHOR]
CALC		Total (will equal 100)
SUM		

C35 Sales Representative Rating

C35	INCLUDE: C10=1-3	RADIO BUTTON GRID. SHOW ROW 4 IF A55 COLUMN C>0
------------	------------------	---

How would you rate the **Client Product** sales representative for the following?

Once you select a response the next attribute will appear. When you have finished rating all the attributes, click the 'Continue' button to proceed. (Select one option)

	Extremely poor						Excellent	Don't Know
	1	2	3	4	5	6	7	9
Disease knowledge	<input type="radio"/>							
Product knowledge	<input type="radio"/>							
Professionalism	<input type="radio"/>							
Support with prior authorizations	<input type="radio"/>							
Visits on a consistent basis	<input type="radio"/>							
Engages in relevant discussions	<input type="radio"/>							
Is well liked/well received by office staff	<input type="radio"/>							

C40 Client Product Overall Manufacturer Execution Rating

C40	INCLUDE: C10=1-3	RADIO BUTTON GRID. SHOW ROW 4 IF A55 COLUMN C>0
-----	------------------	--

How would you rate **Client** for their execution of the **Client Product** launch?

	Extremely poor						Excellent	Don't Know
	1	2	3	4	5	6	7	9
Client for their execution of the Client Product launch	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

C45 Client Product Manufacturer Attribute Rating

C45	INCLUDE: C10=1-3	RADIO BUTTON GRID. SHOW ROW 4 IF A55 COLUMN C>0
-----	------------------	--

How would you rate **Client Product** on the following?

Once you select a response the next attribute will appear. When you have finished rating all the attributes, click the 'Continue' button to proceed. (Select one option)

	Extremely poor						Excellent	Don't Know
	1	2	3	4	5	6	7	9
Hub services/support with prior authorizations	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Key opinion leader endorsement	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Medical science liaison support	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Sales representatives	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
HCP education	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Patient assistance/education program	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Supportive of endocrinology specialty	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Section D: cHI Treatment Perceptions

D10 Overall Product Satisfaction

D10	INCLUDE: ALL	RADIO BUTTON GRID. SHOW ROW 4 IF A55 COLUMN C>0
-----	--------------	--

Generally, how **satisfied** are you with the following **current** treatments for **congenital hyperinsulinism (cHI) patients**?

Once you select a response the next product name will appear. When you have finished rating each product, click the 'Continue' button to proceed. (Select one option)

INSERT PRODUCT FROM LIST BELOW

		Extremely dissatisfied					Extremely satisfied		Don't Know
		1	2	3	4	5	6	7	9
1	Diazoxide	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
2	Somatostatin analogs (SSAs)	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
3	Carb supplements	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
4	Client Product	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
5	SHOW OTHER SPECIFY	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

D15 Current cHI Treatment

D15	INCLUDE: ALL	LOGIC: NUMERIC 0-9999; SUM>S80_2
-----	--------------	----------------------------------

For your current [INSERT S80_2] **diffuse congenital hyperinsulinism (cHI) patients**, how many are **CURRENTLY** treated with the following medications. Since therapies can be taken concomitantly, your allocation can add up to more than [INSERT S80_2] .

As a reminder, you indicated earlier that you currently have [INSERT B20_A] **diffuse congenital hyperinsulinism (cHI) patients** receiving **Client Product**.

Then please estimate how many will be treated with the following medications **6 MONTHS FROM NOW**.

(Please fill in boxes, whole numbers only.)

	# of diffuse cHI patients CURRENT	# of diffuse cHI patients 6 MONTHS FROM NOW	
1	_____	_____	Diazoxide
2	_____	_____	Somatostatin analogs (SSAs)
3	_____	_____	Carb supplements
4	_____	_____	Client Product
5	_____	_____	Other SPECIFY(_____)
	S80_2	S80_2	

D20 Diazoxide Discontinuation

D20	INCLUDE: SHOW IF D15A_1>0	LOGIC: PERCENTAGE 0-100
-----	---------------------------------	----------------------------

On average, what percentage of the **congenital hyperinsulinism (cHI) patients** who you have treated with **diazoxide** have to discontinue the treatment due to the reasons listed below.

	% of of the congenital hyperinsulinism (cHI) patients treated with diazoxide _____	% discontinued diazoxide treatment due to non-response
	_____	% discontinued diazoxide treatment due to partial response
	_____	% discontinued diazoxide treatment due to significant adverse event

Section E: cHI Patient Records

INTRO PAGE – SHOW JUST ONCE

In this section, we would like to ask you about the **diffuse congenital hyperinsulinism (cHI) patients** that you personally treat.

We will ask you to please refer to your medical records/EMR and fill out the patient chart below based on your records.

Please complete this section with the requested patient data using the information provided in each patient's medical record. Please be assured that all patient data requested is fully anonymized and will be collated with data provided by other respondents' and presented to the sponsor in an aggregated and fully anonymized form.

Patient Characteristics/Demographics		
E1	Age INCLUDE: ALL	Please select one: 1. <1 year old 2. 1-3 years old 3. 4-6 Years old 4. 7-18 years old 5. >18 years old 6. Don't know/not applicable
E2	Gender INCLUDE: ALL	Please select one: 1. Female

		<ul style="list-style-type: none"> 2. Male 3. Don't know/not applicable
E3	<p>Insurance coverage INCLUDE: ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1. Commercial health insurance plan (e.g., Aetna, Blue Cross, etc.) sponsored by your employer or that you purchased on your own 2. Medicare 3. Medicaid 4. Federal insurance provided by any of the following: 5. Department of Veterans Affairs (VA) 6. TRICARE Military Health System (MHS) 7. Indian Health Services (IHS) 8. Other federally funded insurance 9. AIDS Drug Assistance Program (ADAP) 10. Don't know/not applicable
E4	<p>Current estimated patient weight INCLUDE: ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1. Significantly below average weight 2. Somewhat below average weight 3. Slightly below average weight 4. Average weight 5. Slightly above average weight 6. Somewhat above average weight 7. Significantly above average weight
E5	<p>Current estimated patient height INCLUDE: ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1. Significantly below average height 2. Somewhat below average height 3. Slightly below average height 4. Average height 5. Slightly above average height 6. Somewhat above average height 7. Significantly above average height
E6	<p>Pancreatectomy Status INCLUDE: SHOW ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1. Almost full pancreatectomy 2. Partial pancreatectomy 3. No pancreatectomy 4. Don't know/not applicable
[NEW SCREEN] Patient Status		
E7	<p>Number of months since diagnosis INCLUDE: SHOW ALL</p>	<p>Please select one [CANNOT BE LONGER THAN B3]:</p> <ul style="list-style-type: none"> 1. <3 months 2. 4-6 months 3. 7-12 months

		<ul style="list-style-type: none"> 4. 13-24 months 5. 25-60 months 6. >60 months 7. Don't know/not applicable
E8	<p>Total office/facility visits in the past 12 months</p> <p>INCLUDE: SHOW ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1. 0 2. 1-2 3. 3-5 4. 6-10 5. >10 6. Don't know/not applicable
E9	<p>Patient level of physical functioning</p> <p>INCLUDE: SHOW ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1 Unable to perform any physical tasks 2 3 4 Able to perform basic tasks with some difficulty 5 6 7 Fully active without limitations ○Don't know/not applicable
E10	<p>Patient level of emotional functioning</p> <p>INCLUDE: SHOW ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1 Severely impaired 2 3 4 Fair 5 6 7 Excellent ○Don't know/not applicable
E11	<p>Patient level of social functioning</p> <p>INCLUDE: SHOW ALL</p>	<p>Please select one:</p> <ul style="list-style-type: none"> 1 Severely impaired 2 3 4 Fair 5 6

		7 Excellent ○Don't know/not applicable
E12	Patient comorbidities INCLUDE: SHOW ALL	Please select all that apply: 1. Intellectual disability and developmental delay 2. Epilepsy and seizures 3. Cerebral palsy 4. Motor and speech delays 5. Vision loss and cortical blindness 6. Macrosomia and overgrowth 7. Feeding difficulties 8. Hepatomegaly 9. Cardiomyopathy 10. Beckwith-Wiedemann syndrome (BWS) 11. Sotos syndrome 12. Kabuki syndrome 13. Hyperinsulinism-hyperammonemia (HI/HA) syndrome 14. Congenital Disorders of Glycosylation (CDG) 15. None 16. Don't know/not applicable
E13	# of hypoglycemia events in the past week INCLUDE: SHOW ALL	_____ # of hypoglycemia events in past week [RANGE: 0-99]
[NEW SCREEN] Current cHI Medication		
E14	Current cHI medication (select all that apply) INCLUDE: SHOW ALL	Please select all that apply: 1. Diazoxide 2. Somatostatin analogs (SSAs) 3. Carb supplements 4. Client Product 5. Other SPECIFY(_____) 6. Don't know/not applicable
E15	How would you classify the patient's response to diazoxide? INCLUDE: E14=1	Please select one: 1. Full response 2. Partial response 3. No response 4. Don't know/not applicable
E16	How would you classify the patient's level of adverse events from taking diazoxide? INCLUDE: E14=1	Please select one: 1. Significant 2. Moderate 3. Minimal

		4. Don't know/not applicable
--	--	------------------------------

Section F: CLIENT PRODUCT TPP Evaluation & Future Prescribing

In this next section, we would like you to review a description of a therapy for the **treatment of congenital hyperinsulinism (cHI)** that **may** obtain FDA approval over the next few years. This hypothetical product description has been drafted for market research purposes.

As you review the description, we'll have you use a highlighting tool to indicate what characteristics will impact your decision whether to prescribe it.

F10 Confirm Assumptions

F10	INCLUDE: SHOW ALL	LOGIC: MULTI-SELECT; MUST CLICK ALL TO MOVE FORWARD DO NOT RANDOMIZE
-----	-------------------	---

When reviewing the hypothetical product description, we would like you to **assume ALL of the following to be true**. Please select each option to confirm.

(Please select each option listed)

- 1 The product has been approved for the treatment of **congenital hyperinsulinism (cHI)** based on the information and clinical data presented in the profile
- 2 The product has been on the market long enough for you to have sufficient clinical experience to know that the product performs as described
- 3 Access and reimbursement would not limit your prescribing

F20 Review Product X (Client Product) TPP

F20	INCLUDE: ALL	LOGIC: DISPLAY PROFILE FOR PRODUCT X HIGHLIGHTER TOOL FOR PROFILE; FORCE RESPONDENT TO SELECT AT LEAST ONE ITEM. ALLOW HIGHLIGHTING FOR INDIVIDUAL BULLETS AS NOTED IN THE DESIGN FILE.
-----	--------------	--

Please review below the hypothetical profile of **Product X**, a therapy in development for **congenital hyperinsulinism (cHI)** and identify what you would consider benefits and drawbacks of this product:

First hover over the different sections, then click either:

Green to indicate that section contains a benefit or potential reason to use **Product X**

Red to indicate that section contains a drawback or a potential reason to not use **Product X**

Note: You do not have to make a selection for all areas.

1	[TPP COMPONENTS PLACEHOLDER] <i>[Note for reader: we will provide separately a copy of the TPP that shows which components/blocks of text will be selectable items in the Highlighter exercise]</i>
---	--

LOGIC	DO NOT FORCE A SELECTION (GREEN/RED) FOR EACH CLICKABLE ITEM; ALLOW SAME SELECTION (GREEN/RED) ACROSS CLICKABLE ITEMS (E.G., ALL ITEMS OR NONE CAN BE GREEN)
-------	--

[DISPLAY PRODUCT X (Client Product) TPP]

[DISPLAY TEXT BELOW PROFILE]

Please be sure you select all the items that may impact your decision whether to prescribe this product.

F30 Product X (Client Product) Perception

F30	INCLUDE: SHOW ALL	LOGIC: SINGLE SELECT
-----	----------------------	-------------------------

How would you rate your **overall perception** of **Product X** as a treatment option for your **congenital hyperinsulinism (cHI)** patients?

To see the **Product X** description again, click [HERE](#) [INSERT LINK TO PRODUCT X]
(Select one)

1	2	3	4	5	6	7
Unfavorable perception						Favorable perception

F35 Product X (Client Product) Risk/Benefit

F35	INCLUDE: SHOW ALL	LOGIC: SINGLE SELECT
-----	----------------------	-------------------------

How would you rate the overall risk/benefit profile of **Product X** as a treatment option for your **congenital hyperinsulinism (cHI)** patients?

To see the **Product X** description again, click [HERE](#) [INSERT LINK TO PRODUCT X]
(Select one)

1	2	3	4	5	6	7
Risk outweighs benefit						Benefit outweighs risk

F40 Product X (Client Product) Advance Over Existing Agents

F40	INCLUDE: SHOW ALL	LOGIC: SINGLE SELECT
-----	----------------------	-------------------------

How much of an advance is **Product X** as a treatment option over other agents to treat your **congenital hyperinsulinism (cHI)** patients?

To see the **Product X** description again, click [HERE](#) [INSERT LINK TO PRODUCT X]
(Select one)

1	2	3	4	5	6	7
Not an advance at all						Substantial advance

F45 Product X Performance on Treatment Drivers

F45	INCLUDE: SHOW ALL	LOGIC: REPEAT QUESTION FOLLOWING MAX-DIFF DESIGN
------------	-----------------------------	--

Next, we would like to ask about the attributes you associate with **Product X** the least and which attributes you associate with **Product X** the most, **based on what you read in the profile.**

You will be evaluating X sets of attributes. For each set of five attributes that you see, please select the box next to the attribute that you associate with **Product X** the least and the attribute that you associate with **Product X** the most.

Associate with Product X the Least		Associate with Product X the Most
<input type="radio"/>	Attribute 1	<input type="radio"/>
<input type="radio"/>	Attribute 2	<input type="radio"/>
<input type="radio"/>	Attribute 3	<input type="radio"/>
<input type="radio"/>	Attribute 4	<input type="radio"/>
<input type="radio"/>	Attribute 5	<input type="radio"/>

Potential List of Attributes

Mechanism of action
Efficacy in patients, regardless of genetics
Speed of efficacy onset
Reduction in hypoglycemia frequency
Risk of edema
Risk of excessive hair growth
Risk of tachycardia
Level of treatment emergent AEs
Risk of discontinuation due to AEs
Mode of administration (e.g. oral vs. IV)
Dosing frequency
Ease of titration
Can be used in combination therapy
Extensiveness of clinical data
Indication for patients as young as three months old

F50 Expected Product X Utilization

F50	INCLUDE: SHOW ALL	LOGIC: NUMERIC; ADD POP UP ERROR IF ROW 5 = 0 <u>AND</u> ANY OF ROWS 1 THRU 4 CHANGE FROM A50: "YOU INDICATE YOU WILL NOT USE THE NEW PRODUCTS, SO YOUR CURRENT ESTIMATES SHOULD NOT CHANGE. PLEASE REVISE YOUR RESPONSES";
-----	----------------------	---

We'd like to understand how, if at all, the [availability of this new therapy \(Product X\)](#) would impact your prescribing approach for congenital hyperinsulinism (cHI) patients.

Thinking about the **congenital hyperinsulinism (cHI)** you treat, if this new treatment option were available, how would your prescribing change for this group?

Your previous responses are shown as a reminder and to help with any estimated changes. If there is no change from your earlier responses, just re-input the same numbers into the second column.

Please click here to review the profile of **Product X** again. [\[INSERT LINK TO PRODUCT X\]](#)
(Please fill in boxes, whole numbers only. Please note your total must sum to 100%.)

		A	B
		# of congenital hyperinsulinism (cHI) patients	
		Current	REVISED with Product X
1	Diazoxide	SHOW D15_1	
2	Somatostatin analogs (SSAs)	SHOW D15_2	
3	Carb supplements	SHOW D15_3	
4	Client Product	SHOW D15_4	
5	SHOW OTHER SPECIFY A50_4	SHOW D15_45	
6	Product X	N/A	
	Total	SUM TO 100%	SUM TO 100%

F55 Reasons to Prescribe Product X

F55	INCLUDE: SHOW IF F50_5>0	LOGIC: DRAG AND DROP, RANK 1-5, FORCE ONE RANK. RANDOMIZE, ANCHOR 95
------------	-----------------------------	--

In rank order (**up to 5**), what are the main reasons you might expect to initiate a patient on **Product X** for your **congenital hyperinsulinism (cHI) patients**?

The top (or most influential) reason to use **Product X** should be placed at the top of the list. Rank at least 1 and up to 5.

Please click here to review the profile of **Product X** again. [INSERT LINK TO PRODUCT X]

(Drag up to 5 items from the left column to the right, and order them with #1 (most influential) at the top.)

- | | Rank up to 5 |
|-----------|---|
| 1 | <input type="checkbox"/> Novel mechanism of action |
| 2 | <input type="checkbox"/> IV infusion |
| 3 | <input type="checkbox"/> Dosed every 2-4 weeks |
| 4 | <input type="checkbox"/> Indicated for patients age 3 months+ |
| 5 | <input type="checkbox"/> Significant improvement in daily time in hypoglycemia |
| 6 | <input type="checkbox"/> Significant reduction in number of hypoglycemia weeks |
| 7 | <input type="checkbox"/> Predictable and dose dependent pharmacokinetics |
| 8 | <input type="checkbox"/> No adverse drug reactions |
| 9 | <input type="checkbox"/> No study terminations |
| 10 | <input type="checkbox"/> No clinically significant hyperglycemia or hyperglycemia AEs |
| 11 | <input type="checkbox"/> Near universal response at top dose |
| 12 | <input type="checkbox"/> Improvement in hypoglycemia time and events up to 90% at top doses |
| 95 | <input type="checkbox"/> Other (Please specify _____) |

F60 Confirm Non-Intent to Use Interim Products

F60	INCLUDE: IF F50_5= 0	LOGIC: SINGLE SELECTION EACH ROW. SHOW ROWS WHERE F50_5 = 0.
-----	----------------------	--

You indicated you would not prescribe the following products for your **congenital hyperinsulinism (cHI) patients**. Is that correct?

Please click here to review the profile of **Product X** again. [INSERT LINK TO PRODUCT X]
(Please select one option for each row.)

REPEAT RANDOMIZATION ORDER OF C10-C30

	A	B
	Correct, I will <u>not</u> prescribe	No, I might prescribe at some point
Product X	<input type="radio"/>	<input type="radio"/>

F65 Reasons to Not Prescribe Product X

F65	INCLUDE: SHOW IF F50_5=0	LOGIC: DRAG AND DROP, RANK 1-5, FORCE ONE RANK. RANDOMIZE, ANCHOR 95
-----	-----------------------------	---

In rank order (**up to 3**), what are the main reasons you **do not** expect to initiate a patient on **Product X** for your **congenital hyperinsulinism (cHI) patients**?

The top (or most influential) reason **not** to use **Product X** should be placed at the top of the list. Rank at least 1 and up to 3.

Please click here to review the profile of **Product X** again. [INSERT LINK TO PRODUCT X]

(Drag up to 3 items from the left column to the right, and order them with #1 (most influential reason NOT to use) at the top.)

Rank <u>up to 3</u>	
1	<input type="checkbox"/> Novel mechanism of action
2	<input type="checkbox"/> IV infusion
3	<input type="checkbox"/> Dosed every 2-4 weeks
4	<input type="checkbox"/> Indicated for patients age 3 months+
5	<input type="checkbox"/> Frequency of treatment emergent AEs 70%
6	<input type="checkbox"/> Lack of long term data
7	<input type="checkbox"/> Efficacy is inferior to current options (diazoxide, sandostatin)
8	<input type="checkbox"/> Can not wean patients off like other therapies
95	<input type="checkbox"/> Other (Please specify _____)

F70 Impact of Product X on Patient Records

F70	INCLUDE: SHOW ALL	LOGIC: OPEN END;
-----	-------------------	------------------

Next, we will refer back to the first patient record(s) you shared earlier and ask you a couple more questions about their treatment.

[SHOW TABLE ON SAME PAGE]

Now assuming that **Product X was available**, what treatment(s) would you be most likely to **prescribe NEXT** if this patient needed to be switched?

Please review the patient description you provided earlier and then identify if you would change how you would treat this patient with **Product X** available.

You may choose to **keep this patient on the same** treatments you selected earlier **or you can change some (or all) of them**.

[ALLOW CHANGES ONLY IF PRODUCT X IS SELECTED]

Please click here to review the profile of **Product X** again. [INSERT LINK TO PRODUCT X]

SHOW ON SAME SCREEN

INCLUDE: SHOW ALL

[HEADER ONLY. SHOW AT THE TOP OF ALL PRF SCREENS]		
Patient # [SHOW LOOP NUMBER]		
Patient Characteristics/Demographics		
F70_1	Age	SHOW E1 RESPONSE
F70_2	Gender	SHOW E2 RESPONSE
F70_3	Insurance coverage	SHOW E3 RESPONSE
F70_4	Current estimated patient weight	SHOW E4 RESPONSE
F70_5	Current estimated patient height	SHOW E5 RESPONSE
F70_6	Pancreatectomy Status	SHOW E6 RESPONSE
[NEW SCREEN] Patient Status		
F70_7	Number of months since diagnosis	SHOW E7 RESPONSE
F70_8	Total office/facility visits in the past 12 months	SHOW E8 RESPONSE
F70_9	Patient level of physical functioning	SHOW E9 RESPONSE
F70_10	Patient level of emotional functioning	SHOW E10 RESPONSE
F70_11	Patient level of social functioning	SHOW E11 RESPONSE

F70_12	Patient comorbidities	SHOW E12 RESPONSE
F70_13	# of hypoglycemia events in the past week	SHOW E13 RESPONSE
[NEW SCREEN] Current cHI Medication		
F70_14	Current cHI medication (select all that apply)	<p>Please select all that apply, now that Product X is available:</p> <ol style="list-style-type: none"> 1. Diazoxide 2. Somatostatin analogs (SSAs) 3. Carb supplements 4. Client Product 5. Other SPECIFY(_____) 6. Product X <p>Don't know/not applicable</p>
F70_15	How would you classify the patient's response to diazoxide? DON'T SHOW	SHOW E15 RESPONSE
F70_16	How would you classify the patient's level of adverse events from taking diazoxide? DON'T SHOW	SHOW E16 RESPONSE

F80 Reasons for Patient Record Selection

F80	INCLUDE: SHOW ALL	LOGIC: OPEN END;
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Please briefly explain your treatment choice.

Please enter your responses below and be as specific as possible.

[_____]

Section G: Physician cHI Attitudes and Demographics

G10. cHI Treatment Attitudes

G10	INCLUDE: ALL	LOGIC: CAROUSEL SCALE 1-7; RANDOMIZE ROWS
-----	--------------	---

Please indicate your level of agreement with each of the statements below.

INSERT STATEMENT FROM LIST BELOW

(Please select one option)

1	2	3	4	5	6	7
Strongly Disagree	Mostly Disagree	Somewhat Disagree	Neither	Somewhat Agree	Mostly Agree	Strongly Agree

ITEMS TO INCLUDE:

- | | |
|----|---|
| 1 | I am more aggressive than most of my peers with respect to prescribing new therapies for the conditions I treat |
| 2 | Insurance companies significantly restrict my optimal use of therapies to treat my patients with the most common conditions |
| 3 | There is a high unmet need for more effective agents for diffuse congenital hyperinsulinism (cHI) |
| 4 | Insurance companies significantly restrict my optimal use of therapies to treat my rare disease patients |
| 5 | There is a high unmet need for safer agents for diffuse congenital hyperinsulinism (cHI) |
| 6 | Safety is just as important as efficacy in deciding which new diffuse congenital hyperinsulinism (cHI) agent is best for my patients |
| 7 | Mechanism of action will heavily influence my selection of therapies for diffuse congenital hyperinsulinism (cHI) in the future. |
| 8 | I am unfamiliar with the negative allosteric modulator of the insulin receptor (INSR) pathway for treating diffuse congenital hyperinsulinism (cHI). |
| 9 | Mode of administration (infusion, subcutaneous injection, etc.) will be very important in my decision for which new diffuse congenital hyperinsulinism (cHI) therapy is the best for my patients. |
| 10 | A therapy that is oral will be my most preferred in the future for diffuse congenital hyperinsulinism (cHI) |
| 11 | I am more likely to use a therapy for diffuse congenital hyperinsulinism (cHI) if it is able to address one or more common comorbidities with cHI. |
| 12 | Estimating what therapy I will prescribe for congenital hyperinsulinism (cHI) in the future is difficult given that there are currently no approved therapies to treat the condition. |
| 13 | A therapy that is IV/infused will be my most preferred in the future for congenital hyperinsulinism (cHI) |

G20 Treatment Priority

G20	INCLUDE: SHOW ALL	LOGIC: RADIO BUTTON SCALE 1-7
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When treating **diffuse congenital hyperinsulinism (cHI) patients**, how do you balance efficacy and safety?

(Please select one option)

1	2	3	4	5	6	7
Efficacy significantly more important	Efficacy somewhat more important	Efficacy slightly more important	Efficacy and safety are equally important	Safety slightly more important	Safety somewhat more important	Safety significantly more important

SAME SCREEN

G30 Explanation of Treatment Priority

G30	INCLUDE: SHOW ALL	LOGIC: OPEN END;
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Please briefly explain your rating regarding how you balance efficacy and safety for **diffuse congenital hyperinsulinism (cHI) patients**.

Please enter your responses below and be as specific as possible.

G40 Number of cHI Patients Treated Cumulatively

G40	INCLUDE: ALL	LOGIC: NUMERIC 0-99;
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You indicated that you currently treat [INSERT S80_2] diffuse congenital hyperinsulinism (cHI) patient(s).

Now please estimate how many diffuse congenital hyperinsulinism (cHI) patient(s) you have cumulatively treated in your medical career. *(Please fill in boxes, whole numbers only.)*

1	Number of patients cumulatively treated in medical career	_____	Diffuse congenital hyperinsulinism (cHI) patient(s)
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G50 Number of Years Treating cHI Patients

G50	INCLUDE: ALL	LOGIC: NUMERIC 0-99;
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You indicated that you currently treat [INSERT S80_2] diffuse congenital hyperinsulinism (cHI) patient(s).

Now please estimate how many years you have been treating congenital hyperinsulinism (cHI) patient(s) y in your medical career. *(Please fill in boxes, whole numbers only.)*

1	Number of years treating	_____	congenital hyperinsulinism (cHI) patient(s)
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G60 Conversations with Caregivers

G60	INCLUDE: ALL	LOGIC: RANDOMIZE. MULTI SELECTION.
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From the list below, please select the types of conversations that you **most often** have with the parents/caregivers of a **diffuse congenital hyperinsulinism (cHI)** infant/child patient **after a diagnosis occurs**. *(Select all that apply.)*

- | | | |
|---|--------------------------|--|
| 1 | <input type="radio"/> | Potential need for pancreas surgery/pancreatectomy |
| 2 | <input type="radio"/> | Potential for diazoxide non-response |
| 3 | <input type="radio"/> | Potential for diazoxide cardiovascular side effects |
| 4 | <input type="radio"/> | Potential for diazoxide edema side effects |
| 5 | <input type="radio"/> | Potential for diazoxide hair growth side effects |
| 6 | <input type="radio"/> | Other (Please specify _____) |
| 7 | <input type="checkbox"/> | Do not have any conversations like these with parents/caregivers |

G70 Practice Type

G70	INCLUDE: ALL	LOGIC: SINGLE SELECTION
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Which of the following best describes your **primary** practice setting?
(Please select one option)

- | | | |
|---|-----------------------|--|
| 1 | <input type="radio"/> | Office-based single specialty |
| 2 | <input type="radio"/> | Office-based multispecialty (<u>not</u> hospital-owned) |
| 3 | <input type="radio"/> | Office-based multispecialty (hospital-owned) |
| 4 | <input type="radio"/> | Community / non-teaching hospital |
| 5 | <input type="radio"/> | Academic / teaching hospital |
| 6 | <input type="radio"/> | HMO |
| 7 | <input type="radio"/> | Other (Please specify _____) |

G80 Infusion Capability

G80	INCLUDE: ALL	LOGIC: SINGLE SELECTION
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Which, if any, of the following facilities or services do you have available for patients at the practice location where you see most of your **diffuse congenital hyperinsulinism (cHI) patients**?

(Select one response per row.)

		A Yes	B No
1	Intrathecal administration	<input type="radio"/>	<input type="radio"/>
2	IV Infusion clinic or capabilities	<input type="radio"/>	<input type="radio"/>
3	Hospital pharmacy	<input type="radio"/>	<input type="radio"/>

G90 Number of Team Members at Location Treating cHI Patients

G90	INCLUDE: ALL	LOGIC: NUMERIC 0-99; RANDOMIZE ROWS
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You indicated that your practice manages [INSERT S80_2] **diffuse congenital hyperinsulinism (cHI) patients** overall. To the best of your ability, please estimate the number of individuals in each role below, at your facility/practice, who assist in managing these **cHI** patients.

(Please fill in boxes, whole numbers only.)

	# of Team Members Involved in Managing cHI Patients	Role/Title
1	_____	Pediatric endocrinologists
2	_____	Endocrinologists
3	_____	Radiologists
4	_____	Pathologists
5	_____	Anesthesiologists
6	_____	Surgeons
7	_____	Advanced practice nurses
8	_____	Nurses
9	_____	Social workers
10	_____	Genetic counselors
11	_____	Psychologists
12	_____	Researchers
13	_____	Speech and feeding therapists
14	_____	Dietitians
15	_____	Other SPECIFY(_____)
16	<input type="radio"/>	Select if you don't know the number of individuals or roles responsible for managing cHI patients at your facility/practice

G100 Professional Time Spent on Managing cHI Patients

G100	INCLUDE: ALL	LOGIC: PERCENTAGE 1-100%
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Please estimate the percentage of your professional time that you spend on treating **diffuse congenital hyperinsulinism (cHI) patients** at your facility/in your practice.
(Please fill in the box, with a percentage between 1 and 100%.)

1 | _____ Percentage of professional time spent managing **diffuse congenital hyperinsulinism (cHI) patients** at your facility/in your practice

GD3 Data Controller Statement

GD3	INCLUDE: SHOW IF S01= 3, 4 OR 5	LOGIC: DISPLAY TEXT ONLY
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SHOW IF S01=3 OR 4 OR 5 (SPAIN OR FRANCE OR GERMANY):

Thank you for kindly contributing to this study.

In keeping with the General Data Protection Regulation, the companies acting in this study as Data Controllers are APEX Market Research Services LLC, the research agency that designed this survey and **XXX**, the firm that sent you the research invitation.

THANK AND CLOSE