

Seeking Community Feedback in the Development of Autoimmune and Immune Mediated CDEs

Advancing the Use and Development of
Common Data Elements in Research
Workshop

March 6-7, 2024

What is IBD?

Inflammatory Bowel Disease is a collection of diseases that affect roughly 1 in 100 Americans. Crohn's Disease and Ulcerative Colitis predominately affect the gut but can manifest across the entire body.

It is an incredibly complex disease with an unknown origin that presents in a various heterogeneous manner with overlapping pathogenesis among other immune mediated diseases.

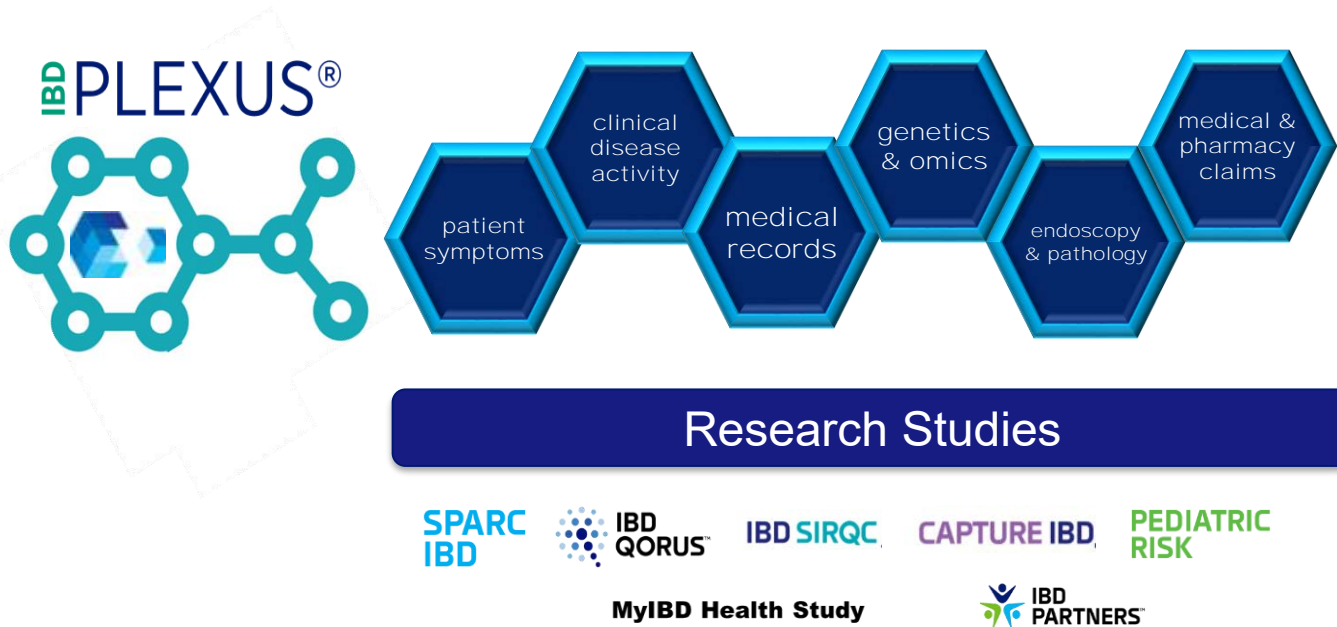
Currently, many treatments exist, however the therapeutic ceiling is incredibly low. Treatment is limited by lack of novel drug mechanisms, a lack of understanding of the disease, insufficient biomarkers, and low access to available medications.

Our Mission

To cure Crohn's disease and ulcerative colitis, and to improve the quality of life of children and adults affected by these diseases.

IBD Plexus

IBD Plexus is a research ecosystem that accelerates progress towards precision medicine by arming our members with integrated clinical and patient-reported outcomes, along with genetic and molecular data with the goal of revolutionizing the care of patients living with IBD.



STRENGTH IN NUMBERS



The Need for CDEs

Heterogeneous diseases with overlapping pathogeneses among other immune mediated diseases

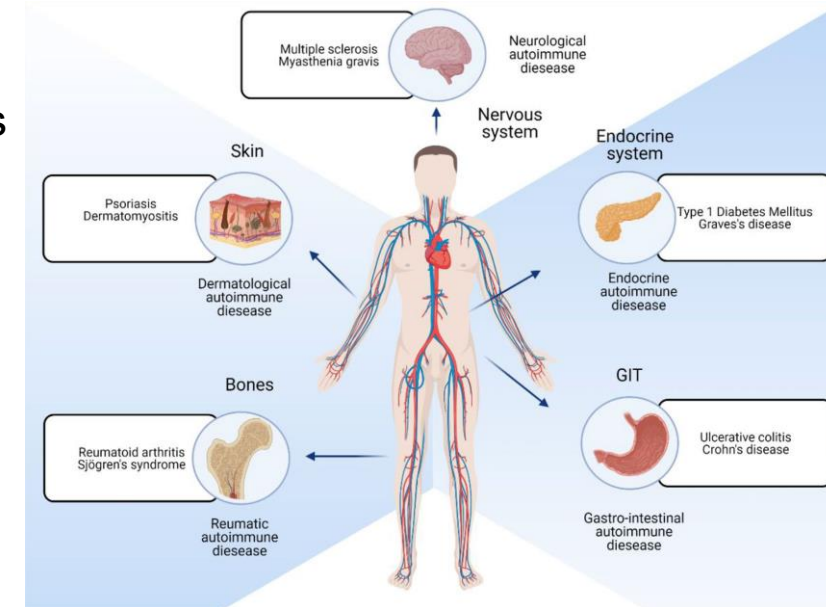
- IBD Patients are more likely to have/develop other immune mediated diseases
 - >25% IBD patients have other immune mediated diseases*
 - No standard definition of disease characteristics across different types of providers (e.g., gastroenterologists, rheumatologists)
 - Limited interoperability across studies/databases
- Insufficient biomarkers

Clinical practices across studies, across sites, across providers vary as well as direct-to-patient settings

- Disease phenotypes
- Medications (biosimilar, dosage, etc.)

Patient experiences vary significantly

- Lifelong journey vs. newly diagnosed
- Flare vs. remission
- Beyond measurable GI symptoms
- Social determinants of health
- Environmental factors



Glover K, Mishra D, Singh TRR. Epidemiology of Ocular Manifestations in Autoimmune Disease. *Front Immunol.* 2021 Nov 2;12:744396

Challenges in CDE Development, Adoption, and Implementation

Individual researcher & individual research study

- Recognizing values of CDEs
- Limited budget, time, and experience
- Available resources

IBD PLEXUS[®] studies and future clinical research

- Collaborative approach
- *Foundation managed CDE development*
- Implementing/providing CDEs and Case Report Forms



Approach in Development of Patient Experience CDEs: Patient Engagement

Patients are experience leaders and each unique voice is important

- They are experts on their own disease experiences.

We seek to involve patients in all aspects of the research cycle

- They are a key piece of research success and have a different perspective than most researchers and physicians.
- By engaging patient early and often we conduct research activities **with** patients *not for* them.
- Identify ways to incorporate patient priorities and increase patient centricity and diversity.

What is Engagement?

SOMETHING YOU FEEL EVEN IF YOU CAN'T DEFINE IT

Engagement should include:

- Having a meaningful connection with an experience and/ or its content
- Feeling a sense of belonging and ownership
- Personal expression, making links with what you know and experience
- Having positive emotions towards an experience
- Purposeful involvement and active contribution
- Perseverance and mastery



Patient Advisory Committee (PAC)

Patient Advisory Committee (PAC)

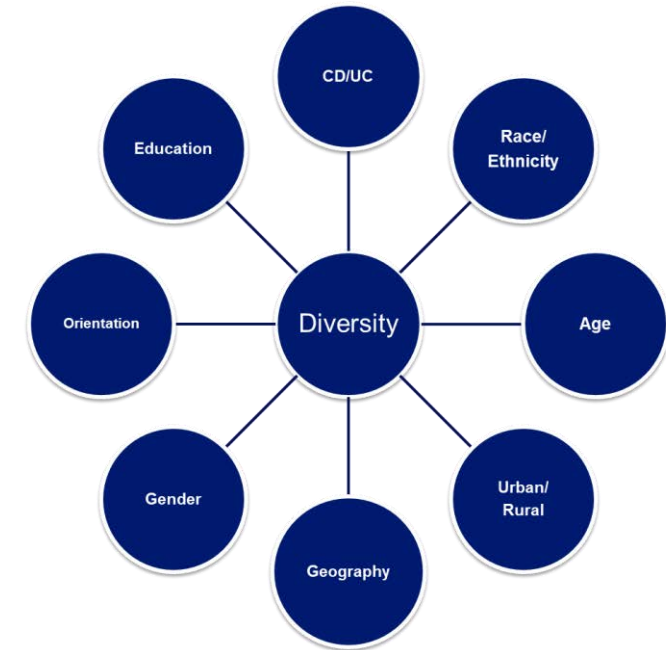
- For each research program we create a diverse PAC to engage patients early and often.
- Diversity is multifaceted in IBD.
- We pay these patients as consultants because their insights are just as important as any other member of the team.
- The patients not only provide feedback, but they also help us **co-create** materials.

Examples of patient facing materials

- CDEs used in surveys
- CDEs describing patient experience and outcome

Goals and impacts

- Language and readability - are patients able to understand your questions and respond options
- Cultural Competency - is this question worded in a way that may be offensive to other groups
- Acceptability - what would make a patient complete this research activity and continue to complete research activities throughout the study



Co-development Use Cases: Sex and Gender

In 2021, the Foundation launched Diversity, Equity, and Inclusion (DEI) initiatives across the organization.

For existing programs and studies, sex and gender were not consistently collected and the terms were used interchangeably.

At that time, there was no validated tools/known CDEs for collecting gender. Either two- or three-part questions were recommended by different institutes.

The Foundation should consider appropriate use of the words sex and gender to avoid confusing terms. Use the term *sex* when reporting biological factors and *gender* when reporting gender identity or psychosocial or cultural factors.

The question wordings and responses were co-developed with patients, researchers, and Foundation. They are now being used in all IBD Plexus studies and across other programs.

What sex were you assigned at birth?

- *Female*
- *Male*
- *Other*

What is your current gender identity?

- *Female*
- *Male*
- *Transgender*
- *Non-binary or Genderqueer*
- *Other*
- *Prefer not to disclose*

Co-development Use Cases: Patient-reported Medication

The Foundation collects patient-report medications in the direct-to-patient study and over-the-counter/supplement surveys.

Examples of what and how patients can report their medications:

- Patients are confident in reporting on their current medications. However, reporting information on biosimilars, frequency, and dosage (especially dosage unit) can vary.
- For frequency, full English wordings are strongly preferred over abbreviations: Once a day; Twice a day; 3 times a day; Once a week; Every 2 weeks; Every 4 weeks; Every 6 weeks; Every 8 weeks; As needed; Not sure; Other
- For dosage, total dosage per dose is preferred over amount plus dosage. (i.e. 100 mg instead of 2 pills of 50 mg)
- For dosage unit, a concentration of infusion therapy or topical cream is harder to report.
- For past medications, reporting exact start date may not be feasible. Options to provide full date or partial date (month-year or year) are preferred.

Dose: What dose of Balsalazide (Colazal, Giazo) do you take each time?

Dose Unit: What is the dose unit of your medication, Balsalazide (Colazal, Giazo)?

Select a dose unit

Frequency: How often are you taking Balsalazide (Colazal, Giazo)?

- Once daily
- Twice daily
- 3 times daily
- Once a week
- Every 2 weeks
- Every 4 weeks
- Every 6 weeks
- Every 8 weeks

 Help

Enter the prescribed dose. This might be more than one pill. For example, if a single dose is two 50mg pills, enter 100mg. Dose units can be entered in the following question.

Co-development Use Cases: IBD-related Symptoms

Patients with Ostomy

Around 10% of Crohn's Disease patients and 30% of ulcerative colitis patients live with ostomy at some point. However, they are often excluded from clinical studies.

The commonly used disease activity indexes do not apply to them since the score components include symptoms related to bowel movement.

- Average Number of Liquid or Soft Stools per Day (over 7 days)?
- Stool frequency
- Rectal bleeding

Response option as *“Not applicable, I have an ostomy.”* is added. More research and development is needed for disease activity indexes for these patients.

Urgency

Bowel urgency is one of the most commonly reported symptoms.

It is not widely included in clinical assessments and clinical studies.

Question and response wordings need to be understandable with same meaning among patients. IBD Plexus studies have adopted a CDE using None to Severe scale along with specific descriptions since 2016.

Urgency Numeric Rating Scale (NRS) was developed in 2022 as 11-point scale ranging from 0 (no urgency) to 10 (worst possible urgency)*.

Stool Urgency

- None. I can wait 15 minutes or longer to have a bowel movement.
- Mild. I need to get to the bathroom within 5-15 minutes.
- Moderate. I need to get to the bathroom within 2-5 minutes.
- Moderately severe. I need to get to the bathroom in less than 2 minutes.
- Severe. Sometimes I am unable to make it to the bathroom in time.
- Not applicable, I have an ostomy

*Dubinsky MC, Shan M, Delbecque L, Lisssoos T, Hunter T, Harding G, Stassek L, Andrae D, Lewis JD. Psychometric evaluation of the Urgency NRS as a new patient-reported outcome measure for patients with ulcerative colitis. J Patient Rep Outcomes. 2022 Nov 5;6(1):114.

Lessons Learned and Recommendations

- Collaborative approach between researchers, Foundation, and patients
 - CDE implementation is a part of research planning and protocol development
 - Minimize burden in data collection when possible
- Co-creation requires developing CDEs with patients and not for patients
 - CDEs for patient experience and patient-reported data should be inclusive, understandable, and well-accepted by patients
- Bringing in the patient voice creates opportunities to better align research with outcomes that matter most to patients
 - Patients find value and stay engaged in study participations

THANK YOU!



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