RTI (h)(s)
Health Solutions

Exploring Heterogeneity in Data
for Demonstrating Product Value

Understand your drug's potential for personalized medicine.

Demonstrate Unknown Product Value

To support your clinical development and commercialization strategy, we can help you identify the people—based on varying comorbidities, genetics, age, gender, physiology, geography, standards of care, or even expectations and preferences—for which your drug is highly effective. And the people for which it isn't. Understanding causes of heterogeneity in patient response and isolating subgroups can help you realize additional benefit from data already collected. Benefits may include the following:

- Increased understanding of treatment effects
- Identification of patients more or less likely to benefit from treatment
- The knowledge to tailor future clinical programs to relevant populations
- Improved inclusion/exclusion criteria to more accurately power trials
- Reduced risk of adverse events
- Informed value messaging
- Additional label claims

Heterogeneity in Clinical Trials and Observational Research

Heterogeneity in treatment response is common, despite designing trials to reduce variability. Additionally, heterogeneity can come from unobserved sources where the cause of variability in response is not known before conducting the study. This can result in an apparent lack of treatment efficacy or minimal statistical difference between treatment arms, as illustrated in Figure 1.

Figure 1:

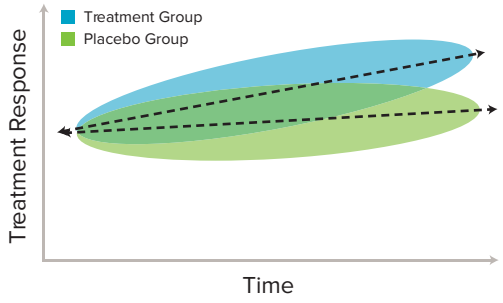
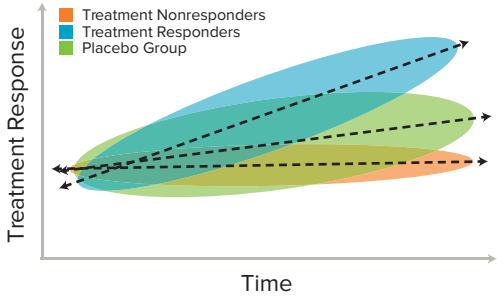


Figure 2:



Uncover and Understand Heterogeneity in Patient Response

Using innovative analytical methods, such as latent growth models (LGMs) and growth mixture models (GMMs), we can help you uncover and understand heterogeneity in patient response. These methods allow patterns within heterogeneous data to emerge from data sources and identify subgroups of patients. Treatment arms of a study may include nonresponders or hyporesponders who can be grouped together in their own subclass. Once identified, hyporesponders can be modeled uniquely. This allows a comparison between the remaining patients for a more accurate assessment of treatment effect between treatment responders and placebo patients. Patients in the hyporesponder subgroup can be studied to determine their unique characteristics, as illustrated in Figure 2.

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Applications

Heterogeneity analysis may be applied to a wide variety of study types. Our experience includes working with data from the following study designs:

- Clinical trials
- Observational studies
- Registries

Our research experts offer conceptual and technical expertise in a range of therapeutic areas and methods used to collect data, including patient-reported outcomes (PROs), clinician-reported outcomes, clinical assessments and laboratory tests, and economic indicators. These analytic methods can be applied to a number of research areas, including outcomes research, health economics, market access, and epidemiology.

Quality Deliverables and Publications

The results from our analyses of your data will lead to many possible quality-assured deliverables:

- Peer-reviewed journal articles
- Abstracts and presentations at professional meetings
- Global value dossier updates
- Internal and external presentations
- Training courses, seminars, and workshops

Selected Publications

Stull DE, McBride D, **Houghton K**, Finlay AY, **Gnanasakthy A**, Balp M-M. Changes in urticaria symptoms, dermatologic-related quality of life, and urticaria-specific quality of life: are they telling us the same thing about response to treatment for chronic spontaneous/idiopathic urticaria (CSU/CIU)? *Allergy*. 2014;69(Suppl s99):122.

Stull DE, Houghton K, Petrillo J. Innovative data analysis for demonstrating product value: analysis of heterogeneity in treatment response in clinical trials. *ISPOR Connections*. 2013 Jan/Feb;19(1):5-8.

Stull DE, Houghton K. Identifying differential responders and their characteristics in clinical trials: innovative methods for analyzing longitudinal data. *Value in Health*. 2013;16(1):164-176.

Stull DE, Wiklund I, Gale R, Capkun-Niggli G, **Houghton K**, Jones P. Application of latent growth and growth mixture modeling to identify and characterize differential responders to treatment for COPD. *Contemp Clin Trials*. 2011 Nov;32(6):818-28.

Stull DE, Vernon MK, Legg JC, Viswanathan HN, Fairclough D, Revicki DA. Use of latent growth curve models for assessing the effects of darbepoetin alfa on hemoglobin and fatigue. *Contemp Clin Trials* 2010;31: 172-79.

Stull DE. Analyzing growth and change: latent variable growth curve modeling with an application to clinical trials. *Qual Life Res* 2008;17(1):47-59.

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