



**WORLD ORPHAN DRUG CONGRESS USA 2019
WEDNESDAY, APRIL 10th, 2019 – 9am to 12pm
Workshop 2 – Hosted by Engage Health**

Identifying and engaging patients and clinicians in the rare disease space

Has the experience of searching for patients with rare diseases, or for the health care providers who care for them, caused you to feel like you were looking for the proverbial needle in a haystack? If so, then this workshop is for you!

Advancing therapies for rare diseases requires a strong community comprised of patients, patient organizations, pharmaceutical companies and health care providers. Introducing new patients to the rare disease community is challenging. To begin with, the path to diagnosis is difficult, sometimes taking as long as 9 years, and often involving an average of 7.3 specialists¹. Once diagnosed, some families join a patient organization. Others become advocates at the state and national level. Some turn their pain into action by founding patient organizations for diseases that have none. Patients and their families who engage in these activities become part of the small but mighty rare disease community.

When a new therapy or a clinical trial is available, patients who are already part of the rare disease community are relatively easy to reach. But what about the ones who do not know there is a rare disease community? What about those who know but do not seek out community involvement? What about those who are lost to expert follow up? Many patients and their families obtain a diagnosis, are told there is nothing to be done, and are sent home to be cared for by their local health care provider. How do we find these patients? How do we find the health care providers who are treating them? How do we reach them with potentially life-saving or life-improving therapies before it is too late?

Hosted by Engage Health, Inc., this three-hour workshop will introduce new tools to engage patients and clinicians at every stage of drug development. Together we can find those needles in that haystack and speed the development of life-improving and life-saving therapies. Together we can make a difference to the patients and families impacted by rare diseases.

1 Engel PA, Bagal S, Broback M, Boice N. PHYSICIAN AND PATIENT PERCEPTIONS REGARDING PHYSICIAN TRAINING IN RARE DISEASES : THE NEED FOR STRONGER EDUCATIONAL INITIATIVES FOR PHYSICIANS. J Rare Disord. 2013;1(2):1-15. <http://www.journalofrareorders.com/pub/IssuePDFs/Engel.pdf>. Accessed December 13, 2018.

AGENDA

Time	Topic	Moderator & Panelists
9:00-9:10 (10 minutes)	Welcome and introduction	Jordan Abhold , Project Manager, Engage Health
9:10-9:20 (10 minutes)	The challenges: identifying and engaging “those who matter”	Bridget Knisely , Project Manager, Engage Health
9:30-10:00 (40 minutes)	Overcoming the challenges when there is no “magic bullet”	Patti Engel , President & Chief Executive Officer, Engage Health



10:00-10:15 (15 minutes)	Data driven approaches	Jaci Schmidt , Database Administrator, Engage Health
10:15-10:30 (15 minutes)	Data driven results in patient advocacy	Christina Hartman , Senior Director, Advocacy & Policy, EveryLife Foundation
10:30 – 11:00	Networking coffee break	
11:00-11:15 (15 minutes)	Data driven results for understanding patient voice	Nita Patel , Senior Director of Patient and Professional Advocacy, Amicus Therapeutics
11:15-11:30 (15 minutes)	Data driven results in increasing diagnosis and research participation	Patty Sibley , Senior Medical Science Liaison, Enzyvant Therapeutics
11:30-12:00 (30 minutes)	Panel: How data driven tools can help speed drug development	Moderator: Jordan Abhold , Project Manager, Engage Health Panelists: Patti Engel , President & Chief Executive Officer, Engage Health Christina Hartman , Senior Director, Advocacy & Policy, EveryLife Foundation Patty Sibley , Senior Medical Science Liaison, Enzyvant Therapeutics Nita Patel , Senior Director of Patient and Professional Advocacy, Amicus Therapeutics