



**Patient
Engagement
Advisory
Committee**

**Connected and
Empowered Patients**

FDA

November 15, 2018

Clustering of SCAD patients leads to novel medical research with Mayo Clinic



Connecting on Inspire: “There is no research because there isn’t anything to research. You either die, need a heart transplant, or are lucky like you.” **When Katherine Leon refused to believe that there were no other people like her- a very rare survivor of SCAD, Spontaneous Coronary Artery Dissection - she started sharing her story on Inspire. She found others like her and researched their patient stories.** Empowered by the Inspire community, she and a fellow advocate attended a symposium at Mayo Clinic and showed their findings to Dr. Sharonne Hayes.

Most rare disease patients search for years and visit many doctors before they are properly diagnosed.

This patient-initiated research led to the community of **50 SCAD** patients being studied **and, along with other advocacy and research efforts, led in February 2018 to the AHA’s first scientific statement on SCAD.**

ONE of many thousands of different rare diseases: Beyond SCAD, many other rare disease patients and caregivers (350k+ across Inspire) are sharing and connecting with each other just like Katherine Leon. They are using Inspire and other ePlatforms as part of their diagnostic odyssey trying to crowdsource clues to their disease, as well as learning how to best cope with and manage it.



Traditional Research Methods Turned Upside Down: The Untapped Potential of “Patient- Initiated” Research in the Study of Rare Diseases

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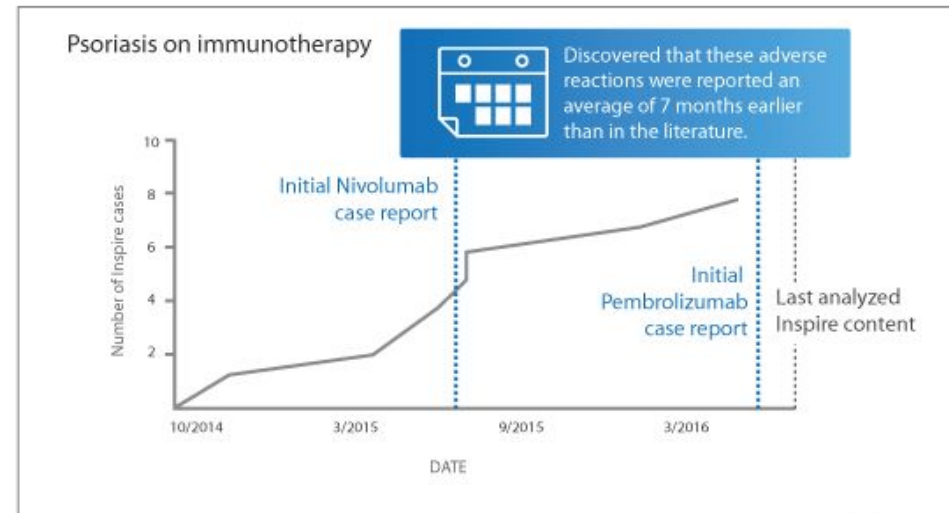
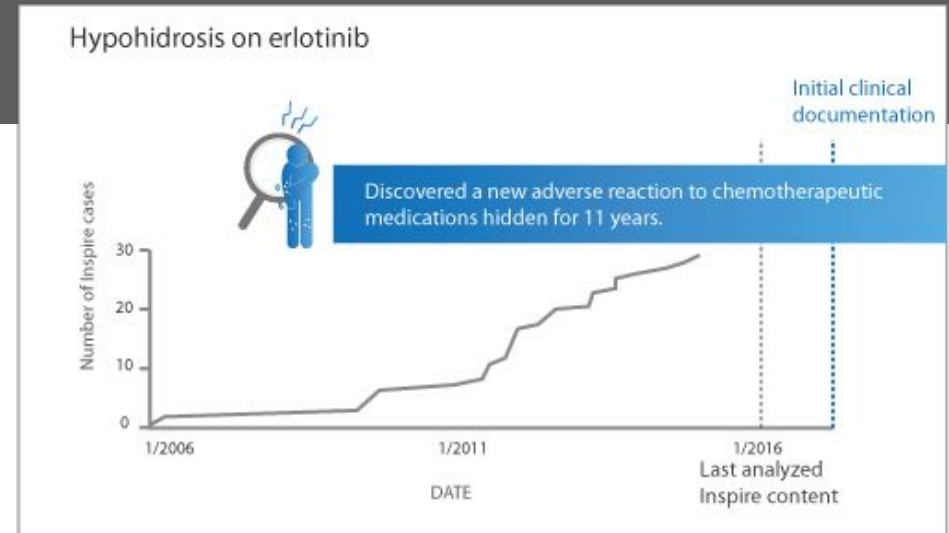
Examples of Rare conditions	Inspire Members in U.S.	% of U.S. population
Ehlers-Danlos	41,022	59%
Von-Hippel Lindau	2,069	35%
PAH	5,279	33%
Pulmonary Fibrosis	17,154	29%
ALS	3,923	24%
Wilson Disease	2,323	23%
Leukodystrophy	2,286	17%
Neurofibromatosis	16,290	16%
Glioblastoma	377	12%

Detecting Chemotherapeutic Skin Adverse Reactions in Social Health Networks Using Deep Learning

Results / Discussion: Several hundred million individuals discuss health-related issues in online forums, offering a robust resource for drug safety surveillance. Our deep learning pipeline extracted mentions of cutaneous ADRs with high precision from organic text on Inspire, detecting ADRs with an average 7-month lead-time from clinical reports. In addition, it uncovered a novel cutaneous ADR, hypohidrosis, not previously reported. We demonstrated the capacity of deep learning-based methods to detect ADRs from online health forums, **offering the potential for real-time pharmacosurveillance with rapid discovery of ADRs preceding FDA detection and published clinical reports.**

Publication

Ransohoff, Julia D., et al. "Detecting Chemotherapeutic Skin Adverse Reactions in Social Health Networks Using Deep Learning." *JAMA Oncology*, vol. 4, no. 4, 2018, p. 581., doi:10.1001/jamaoncol.2017.5688.



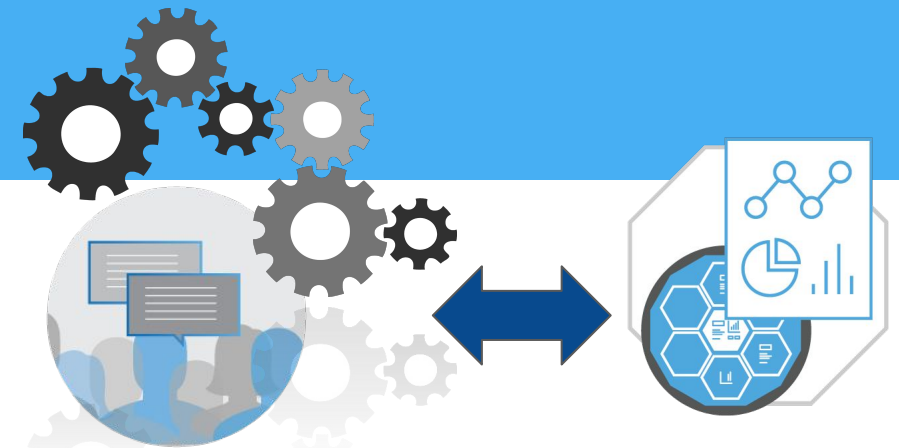
What matters to patients impacted by rare disease?

Partners: FDA CDER, Inspire

Objective: Compare content from in-person Voice of the Patient rare disease event with patient-generated social media community communication. Explore whether online social media reflect what can be obtained in person from patients. Determine the similarities and differences of the results.

Method: 27 patients contributed to FDA Voice of Patient original data. Gathered 880k posts from Inspire members In an iterative process - moving between NLP, manual curation and sociolinguistic analysis -from 880K to 1813 posts- unstructured user generated content on Inspire was effectively extracted, gleaned and thematically organized providing vital information on patient and caregiver experiences, perceptions and perspectives.

Results: A subset of **Inspire patients did not realize that their rare condition was fatal.** FDA VOP participants focused their discussion on the importance of receiving early treatment. **Inspire patients believed that they could/should wait until they felt bad or had symptoms. They did not understand that urgency was essential.** The larger data sample of Inspire UGC helped researchers understand the meaning of quality of life changes-it is not that QoL is affected but **how much it is affected that matters.**



Inspire's posts contained substantially more details-**in the moment- as the symptoms or experiences happened** - about rare disease experience compared with FDA VOP. Analyzing unstructured health conversations on social media while respecting patient privacy, can capture a **wide range of authentic voices that are demographically diverse in age, location, race and gender, across the entire patient journey.** Moreover, this type of analysis is **inclusive of the voices of people who may be unable, because of any number of barriers, to participate in traditional research.**

Publication:In process