

Groundbreaking Research Confirms Need for Medical Education on Rare Diseases

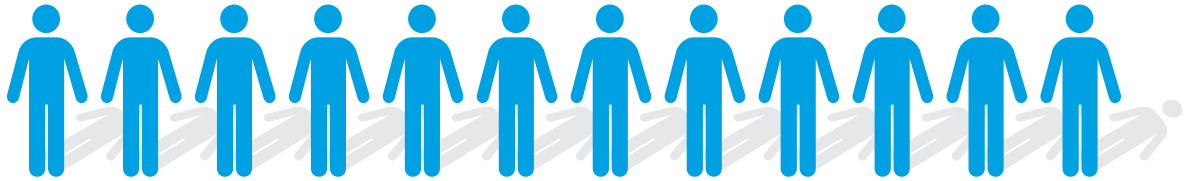
THE CHALLENGES OF

Diagnosing and Treating Patients with Rare Diseases

PERSPECTIVES FROM HEALTHCARE PROFESSIONALS

88% of HCPs*

agree there is a need for more professional content on rare diseases.



HCPs have an average of **12 patients** who have either been diagnosed with, are being treated for, or are suspected to have but remain undiagnosed with a rare disease.



75% of PCPs are treating patients with rare diseases and **11%** are treating more than **5 patients**.



64% of NP/PAs are treating rare disease patients, **21%** are treating more than **5 patients**.

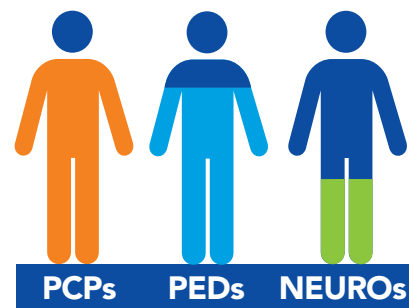
79% of PEDs are treating rare disease patients, **34%** are treating more than **5 patients**.



86% of PCPs refer the patient to a specialist once a rare disease is diagnosed and **71% of PEDs** refer the patient. Only **30% of NEUROs** refer patients upon diagnosis.



89% of NEUROs are treating rare disease patients, **34%** are treating more than **10** and **19%** are treating **5-10 patients**.



*Source: Frontline Medical Communications Research Study: *The Challenge of Diagnosing and Treating Rare Diseases*, January 2014. Base: 1,632 respondents.

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SOURCES AND CHALLENGES SURROUNDING ACCESS TO INFORMATION

In the last **12 months**, to gain a better understanding of diagnosing and treating rare diseases, **HCPs**:

78% searched and assessed the medical literature (e.g. MedlinePlus, PubMed)



69% discussed with local colleagues



CHALLENGES SURROUNDING ACCESS PRESENT OPPORTUNITIES

35% Lack of **networking** opportunities or access to experts related to specific rare diseases



33% Lack of means to **collaborate with colleagues** with knowledge/experience in diagnosis and/or treatment



34% Lack of **awareness** among their professional specialty



30% Lack of proper **training and education**

33% Lack of professional **clinical information**



30% Lack of means to **coordinate care** with other clinicians handling the same case.



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PERSPECTIVES FROM HEALTHCARE PROFESSIONALS

PREFERENCE FOR RESOURCES, AND EDUCATIONAL TACTICS

75% Single-topic or multi-topic medical journals online most valuable



73% Disease specific Symposia at medical conferences

88% of HCPs agree there is a need for more professional content on rare diseases. Respondents rated the following educational tactics as extremely/very valuable to best inform them.



67% On-demand audio/video (webcasts, webinars, audiocasts, etc.)

74% Disease-state websites/multimedia resource centers extremely/very valuable



63% Single-topic or multi-topic medical journals in print



63% Expert Key Opinion Leaders (KOL) speaker programs

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PERSPECTIVES FROM HEALTHCARE PROFESSIONALS

- OPPORTUNITIES** : **91%** value access to free CME/CE credits.
 : **54%** value free CME/CE credits from Frontline's journals and affiliated Websites
 : **34%** value online only

Sources typically used to earn **CME/CE credit**:

76% Attendance at meetings — Local conferences, symposia, events



44% Online webcasts/Podcasts



69% Attendance at meetings — National conventions



39% Medical Journals accessed via print

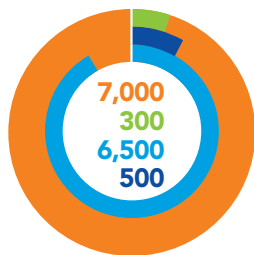
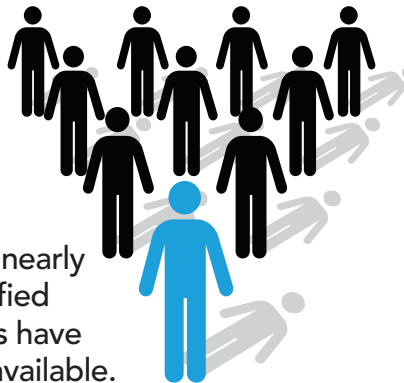
53% Medical Journals accessed via online



22% Audio/Video via CD, DVD, TV

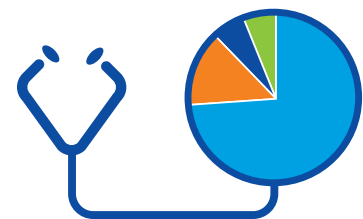


THE MARKET: **1 in 10** patients worldwide and **25-30 million** people in the U.S. has a rare disease. Rare disease is defined as one that strikes fewer than **200,000** patients.



- Only **300** of nearly **7,000** identified rare diseases have treatments available.
- An estimated **500** are common enough to be in any physician's repertoire for diagnosis and **6,500** are very rare.

- Proper diagnosis can take up to **7 years** in the U.S.
- **1,795** potential therapies are in development.



PROFILE OF RESPONDENTS

Physicians	74%
Nurse Practitioners	14%
Physician Assistants	6%
Other healthcare professionals	6%

FRONTLINE
MEDICAL COMMUNICATIONS

PhRMA.org; NIH.gov/NORD.gov; Shire Rare Disease Impact Report 2013; Frontline Medical Communications.
 The Challenge of Diagnosing and Treating Rare Diseases, January 2014. 1,632 respondents.