

M3MI Rare Disease

2026 DIRECTORY

M3 MI is proud to offer the Rare Disease studies, one of the industry's first and most comprehensive syndicated research efforts designed to help healthcare marketers effectively reach an overlooked population and their care providers.



M3 MI

Rare Disease Studies

Connecting Patient, Caregiver, and HCP Perspectives

HCP Rare Disease

A complete view of healthcare providers that treat patients with rare diseases, including the sources they value and information they need across the diagnosis and treatment journey.

Patient & Caregiver Rare Disease

A complete view of rare disease patient and caregiver information needs across the healthcare journey, including in-depth media preferences and behaviors tied to health conditions and treatments.

Together, the studies serve as a well-rounded, detailed lens into the experiences and challenges of living with and treating rare/hard-to-diagnose conditions, paving the way for healthcare marketers to map and optimize patients’ journeys, identify diagnostic barriers and misdiagnosis patterns, and accelerate treatment initiation to support HCPs and drive meaningful impact.

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Common Content

The following pages serve as a topline overview of the conditions and other survey content asked to patients and/or caregivers and/or healthcare providers (HCPs).

Measured Conditions

Conditions by therapeutic area	 Patients	 Caregivers	 HCPs
Autoimmune/Immune Disorders			
ANCA Associated Vasculitis	☒		☒
Axial Spondyloarthritis (axSpA)	☒	☒	☒
Bullous Pemphigoid (BP)	☒		☒
Chronic Granulomatous Disease (CGD)		☒	☒
Chronic Spontaneous Urticaria (CSU)	☒	☒	☒
Crohn's Perianal Fistulas (CPF)	☒		☒
Eosinophilic Esophagitis	☒	☒	☒
Giant-Cell Arteritis	☒		☒
Graves Ophthalmopathy	☒		☒
Hereditary Angioedema (HAE)	☒	☒	☒
IgA Nephropathy (IgAN) or Focal Segmental Glomerulosclerosis (FSGS)	☒	☒	☒
Interstitial Lung Disease/Idiopathic Pulmonary Fibrosis	☒		☒
Primary Biliary Cholangitis/Cirrhosis	☒		☒
Primary Immunodeficiency (PID)		☒	☒
Prurigo Nodularis	☒		☒
Systemic Lupus Erythematosus (SLE)	☒	☒	☒
Blood Disorders			
Beta Thalassemia / Hemoglobinopathy		☒	☒
Hemophilia A or B	☒	☒	☒
Immune Thrombocytopenia	☒	☒	☒
Paroxysmal Nocturnal Hemoglobinuria (PNH)	☒		☒
Primary Systemic Amyloidosis	☒		☒
Sickle Cell Disease	☒	☒	☒
Thrombotic Thrombocytopenic Purpura	☒		☒

Measured Conditions (continued)



Patients



Caregivers



HCPs

Conditions by therapeutic area	Patients	Caregivers	HCPs
Cancer (Oncology) — Blood and Bone Marrow			
Acute Lymphoblastic Leukemia (ALL)		☒	☒
Acute Myeloid Leukemia (AML)	☒	☒	☒
B-Cell Precursor Acute Lymphoblastic Leukemia			☒
Chronic Lymphocytic Leukemia (CLL)	☒	☒	☒
Chronic Myeloid Leukemia (CML)	☒	☒	☒
Diffuse Large B-Cell Lymphoma (DLBCL)	☒	☒	☒
Hodgkin's Lymphoma (HL)	☒		☒
Indolent B-Cell Non-Hodgkin Lymphoma (NHL)	☒	☒	☒
Multiple Myeloma	☒	☒	☒
Myelodysplastic Syndromes (MDS)		☒	☒
Myelofibrosis	☒	☒	☒
Cancer (Oncology) — Solid Tumor			
Brain and Central Nervous System (CNS) Tumors	☒	☒	☒
Desmoid Tumors/Aggressive Neurofibromatosis	☒		☒
Endometrial Cancer	☒		☒
Esophageal Cancer	☒	☒	☒
Gastric (Stomach) Cancer	☒	☒	☒
Gastroenteropancreatic Neuroendocrine Tumors	☒	☒	☒
Glioblastoma	☒	☒	☒
Kaposi Sarcoma	☒		☒
Malignant Pleural Mesothelioma (MPM)	☒	☒	☒
Merkel Cell Carcinoma (MCC)		☒	☒
Pediatric Low-Grade Glioma (pLGG)			☒
Other Pediatric Cancer		☒	☒
Primary Peritoneal Cancer	☒	☒	☒
Renal Cell Carcinoma	☒	☒	☒
Small Cell Lung Cancer (SCLC)	☒	☒	☒
TNBC (Triple-Negative Breast Cancer)	☒	☒	☒
Urothelial Carcinoma	☒	☒	☒

Measured Conditions (continued)



Patients



Caregivers



HCPs

Conditions by therapeutic area	Patients	Caregivers	HCPs
Cardiac Disorders			
Dilated Cardiomyopathy	☒	☒	☒
Hypertrophic Cardiomyopathy	☒	☒	☒
Pulmonary Arterial Hypertension (PAH)	☒	☒	☒
Transthyretin Amyloid Cardiomyopathy (ATTR-CM)	☒	☒	☒
Other Cardiomyopathy			☒
Growth Disorders			
Achondroplasia			☒
Acromegaly			☒
Cystic Fibrosis		☒	☒
Growth Hormone Deficiency		☒	☒
Prader-Willi Syndrome (PWS)		☒	☒
Infectious Diseases			
Meningitis B	☒		☒
Metabolic Disorders			
Duchenne Muscular Dystrophy		☒	☒
General Kidney/Chronic Kidney Disease	☒		☒
Hereditary Transthyretin-Mediated Amyloidosis with Polyneuropathy (ATTRv-PN)	☒		☒
Hypoparathyroidism	☒	☒	☒
Hypophosphatasia		☒	☒
NASH/MASH/MASLD	☒		☒
Nephropathic Cystinosis			☒
Niemann-Pick Disease Type C	☒	☒	☒
Ornithine Transcarbamylase (OTC) Deficiency		☒	☒
Osteogenesis Imperfecta (OI)	☒	☒	☒
Primary Hyperoxaluria Type 1 (PH1)		☒	☒
Primary Mitochondrial Myopathy			☒
Thymidine Kinase 2 Deficiency (TK2D)			☒
Transthyretin Amyloidosis (ATTR)	☒		☒
Wilson Disease	☒	☒	☒

Measured Conditions (continued)



Patients



Caregivers



HCPs

Conditions by therapeutic area	Patients	Caregivers	HCPs
Neurological Disorders			
Bipolar (BD-1 & BD-2)	☒	☒	☒
Chronic Inflammatory Demyelinating Polyneuropathy (CIDP)	☒		☒
Dravet Syndrome	☒	☒	☒
Friedreich's Ataxia (FA)	☒	☒	☒
Huntington's Disease	☒	☒	☒
Idiopathic Hypersomnia	☒	☒	☒
Lambert-Eaton Myasthenic Syndrome (LEMS)	☒		☒
Lennox-Gastaut Syndrome	☒	☒	☒
Metachromatic Leukodystrophy (MLD)			☒
Multiple Sclerosis	☒	☒	☒
Myasthenia Gravis	☒		☒
Narcolepsy	☒	☒	☒
Neuromyelitis Optica Spectrum Disorder (NMOSD)	☒	☒	☒
Neurotrophic Keratitis	☒		☒
Parkinson's Disease	☒		☒
Spinal Muscular Atrophy (SMA)		☒	☒

Content Overview

Topline overview of survey topics asked to patients and/or caregivers and/or HCPs. Question or response list wording may vary based on the study audience. Use the links above the tables to jump to the complete detailed content information for each study:

[Patient & Caregiver Study Content](#)
[HCP Study Content](#)


Topic	Patients	Caregivers	HCPs
Demographics			
Basic respondent information	☒	☒	☒
Expanded respondent demographics (e.g., education level, income, household size)	☒	☒	
Patient Profile (Condition & health detail)			
Stage in patient's journey condition	☒	☒	
Patient health status & symptom severity	☒	☒	
Impact of condition on patient's daily activities	☒	☒	
Patient confidence	☒		
Whether patient has a caregiver	☒		
Home Assistance & Caregiving			
Relationship between patient/caregiver & whether they live in the same household	☒	☒	
Caregiver support activities & involvement in medical decisions	☒	☒	
Age of patient/care recipient (including minors)		☒	
Challenges faced as a caregiver & caregiver confidence		☒	
Social Determinants of Health			
Challenges related to respondent's personal health conditions	☒	☒	
Physician Profile (Professional & practice detail)			
Professional designation			☒
Practice details (e.g., location, Center of Excellence or Specialty Care Center, treats pediatric vs. adult patients)			☒
Number of patients seen & number of prescriptions written in an average week			☒
Drug/device/procedure adoption			☒
Rare disease patient profile (e.g., percent of practice's total patients, activities performed)			☒
Level of influence (e.g., KOL & professional activities, DOL & digital activities)			☒

Content Overview (continued)

[Patient & Caregiver Study Content](#)
[HCP Study Content](#)


Topic	Patients	Caregivers	HCPs
Diagnosis Journey			
Time until diagnosis with rare condition	☒	☒	☒
Ever misdiagnosed	☒	☒	☒
Time from misdiagnosis until correct one	☒	☒	
How found doctor	☒	☒	☒
Testing required for diagnosis	☒	☒	
Barriers to diagnosing patient with rare condition			☒
Clinical information journey (sources for investigating a suspected rare condition)			☒
Treatment Journey & Patient-HCP Interaction			
Patient treatment status	☒	☒	
Current treatments used	☒	☒	
Time from diagnosis to treatment start	☒	☒	
Share of decision-making for treatment (between patient/caregiver and HCP)	☒	☒	☒
Attitudes about HCP primarily treating condition	☒	☒	
Type of healthcare facility	☒	☒	☒
Reasons for stopping or changing treatment	☒	☒	☒
Usefulness of specific treatment/diagnosis aids			☒
Sources of evidence that influence off-label prescribing			☒
Usefulness of patient educational resources			☒
Treatment Impact & Cost			
Impact of current treatment regimen on patient's daily life	☒	☒	
Attitudes about current treatment regimen	☒	☒	
Patient's type of insurance	☒	☒	
How prescriptions are purchased	☒	☒	
Actions taken to help pay for treatment	☒	☒	
Stress due to cost concerns	☒	☒	
Cost considerations when prescribing			☒

Content Overview (continued)

[Patient & Caregiver Study Content](#)
[HCP Study Content](#)


Topic	Patients	Caregivers	HCPs
Health Information Needs & Resources			
Sources of rare condition information used	☒	☒	☒
Information needed along patient journey	☒	☒	☒
Reasons for trusting information sources	☒	☒	
Treatment resources relied on			☒
Preferred content formats			☒
Best platforms and websites for rare disease research			☒
Online Health & Advertising			
Attitudes about online health/condition resources	☒	☒	
Percent of internet time spent consuming health/medical information	☒	☒	
Health-related online activities	☒	☒	
Where seen/heard healthcare advertising & actions taken as a result	☒	☒	
Attitudes about pharma & advertising	☒	☒	
Use of AI for Health or Professional Purposes			
AI platforms/tools used & important features when using for rare condition or treatment information	☒	☒	
AI-powered activities in practice & concerns			☒
Extent to which AI influences patients			☒
Frequency patients bring health information found online/via AI to appointments			☒
Attitudes about AI	☒	☒	☒
Social Media			
Impact of social media influencers or experts	☒	☒	☒
Usage & frequency of professional-only social media sources to gather advice for difficult cases			☒
Percent of internet time spent on social media	☒	☒	
Social media platforms & patient community websites used	☒	☒	

Content Overview

(continued)

[Patient & Caregiver Study Content](#)

[HCP Study Content](#)



Topic	Patients	Caregivers	HCPs
Media Usage			
Hours spent watching TV	☑	☑	
Proportion of personal TV viewing by method (e.g., Smart TV, streaming app or browser on a laptop/mobile device)	☑	☑	
Hours spent listening to radio, streaming music, or podcasts & types of audio listened to	☑	☑	
Hours spent online in a typical day	☑	☑	
Percent of internet time using a mobile device	☑	☑	
Hours spent watching online video	☑	☑	
Time spent with media (8 categories) in quintiles	☑	☑	

Physician Specialties

HCPs were asked about their own specialty and/or subspecialty, and patients and caregivers were asked about the type of HCP that 1) diagnosed the rare condition, and 2) they primarily see for condition management.

Notes: The list below only includes specialties with ≥ 5 respondents in the final survey intab. The specialty applies to all three respondent types (HCP, patient, caregiver) unless otherwise indicated with an icon (refer to icon key).

PRIMARY SPECIALTIES & KEY SUBSPECIALTIES

- ♦ Allergy/Immunology
- ♦ Cardiology
 - General Cardiology
 - Echocardiography 🏥💰
 - Heart Failure & Transplant 💰
 - Interventional Cardiology 💰
 - Nuclear Cardiology 💰
- ♦ Dermatology (General) 🏥💰
- ♦ Diabetes/Endocrinology 💰
- ♦ Gastroenterology (General)
- ♦ Genetics/Genomic Medicine 🏥👨‍👩‍👧‍👦
- ♦ Oncology & Hem/Onc
 - No Subspecialty
 - Breast Oncology 💰
 - Dermatologic Oncology 💰
 - Gastrointestinal Oncology 💰
 - Genitourinary Oncology 💰
 - Radiation Oncology 🏥👨‍👩‍👧‍👦
 - Other Subspecialty 💰
- ♦ Hepatology 💰
- ♦ Infectious Disease 💰
- ♦ Nephrology
- ♦ Neurology
 - General Neurology
 - Clinical Neurophysiology 💰
 - Epileptology 💰
 - Headache 💰
 - Neuromuscular Medicine 💰
 - Sleep Medicine
 - Vascular Neurology (Stroke) 💰
 - Other Subspecialty 💰

Icon Key:

- ♦ 🏥 = at least 5 patients were diagnosed by or are now primarily seen by this specialty
- ♦ 👨‍👩‍👧‍👦 = at least 5 caregivers report their loved one was diagnosed by or is now primarily seen by this specialty
- ♦ 💰 = at least 5 are HCPs who see rare disease patients

- ♦ Ophthalmology 🏥💰
- ♦ Orthopedic Surgery 💰
- ♦ Otolaryngology (ENT) 💰
- ♦ Pediatrics
 - General Pediatrics 💰
 - Allergy/Immunology 💰
 - Endocrinology 💰
 - Hematology 🏥👨‍👩‍👧‍👦
 - Other Specialty 💰
- ♦ Primary Care
- ♦ Psychiatry
- ♦ Pulmonary Diseases/Pulmonology
- ♦ Rheumatology



HCP Study

This includes all content asked in the HCP Study.

Notes: Topics that were asked in the HCP study **only** are indicated by the HCP icon (💰). Topics with no icon were asked in both the HCP and Patient & Caregiver studies, but details in this section reflect how questions were asked to HCPs.

BASIC DEMOGRAPHICS

Age & Generation

Gender

Ethnicity & Hispanic/Latino/Spanish

💰 PROFESSIONAL & PRACTICE PROFILE

Professional designation — Physician, Physician Assistant/Associate (PA), or Nurse Practitioner (NP)

Practice location area — Urban, Suburban, or Rural

Work within a Center of Excellence (COE) or Specialty Care Center

Treats pediatric patients — Exclusively, Both, or No (adults exclusively)

Number of patients seen (in-person/remote) in an avg. week

Number of prescriptions written in an avg. week

Adoption of new drugs/devices/procedures

- ♦ Early Adopters – As soon as it is released/available
- ♦ Early Majority – After a few others have tried it successfully
- ♦ Late Majority – Once it is in fairly common use
- ♦ Traditionalist – After it becomes a standard drug, device, or procedure

Percentage of patients in practice that are diagnosed with a rare condition

“Treater” or “Referrer/Manager” (of rare disease patients)

Activities engaged in with rare condition patients

- ♦ Attempted to make an initial diagnosis
- ♦ Referred the patient to a specialist for diagnosis
- ♦ Treated the rare disease with an FDA-approved medication
- ♦ Treated the symptoms with an off-label or palliative treatment
- ♦ Enrolled or participated in a clinical trial for a rare disease
- ♦ Managed the long-term care of the patient
- ♦ Provided care coordination with other specialists or providers



💰 LEVEL OF INFLUENCE

Frequency consulted by other HCPs for opinions regarding treatment

Key Opinion Leader (KOL)

Professional activities engaged in (last 12 months)

- ♦ Author an article for a publication
- ♦ Review articles for a peer-reviewed publication
- ♦ Member of publication editorial/advisory board
- ♦ Participate on a pharmaceutical advisory board
- ♦ Teach at a medical school
- ♦ Speak at a local professional association meeting (in-person or remote)
- ♦ Speak at a national conference (in-person or remote)
- ♦ Speak at a medical-oriented dinner meeting (in-person or remote)
- ♦ Board membership of a local medical association
- ♦ Board membership of a national medical association

Digital Opinion Leader (DOL)

Work-related digital activities (last 12 months)

- ♦ Authoring a professional newsletter (e.g., Substack) focused on my specialty
- ♦ Creating video-based educational content (e.g., YouTube Med, TikTok Health, professional websites)
- ♦ Hosting or being a regular guest on a medical podcast
- ♦ Moderating discussions in verified provider-only forums (e.g., Sermo, Doximity)
- ♦ One-time guest on a health-related webinar or podcast
- ♦ Publishing clinical ‘pearls’ or trial summaries on LinkedIn/X/Threads
- ♦ Responding to medical questions on social media platforms

Intended audience of DOL activities — Patients, HCPs, or Both

HCP Study (continued)

RARE DISEASE DIAGNOSIS JOURNEY

⌘ Typical age of onset for condition — Adult or Pediatric

Length of time from patient's first visit to receiving confirmed diagnosis (on average)

Percentage of patients with previous misdiagnoses

How found by rare condition patients

- Directly via insurance provider directories, online search, etc.
- Referred by another patient or caregiver
- They are among my regular patients (e.g. primary care or pediatric population)
- Referral from adult primary care providers
- Referral from pediatricians, OB-GYNs, antenatal, or neonatal specialists
- Referral from other specialists
- Via patient advocacy groups or associations

⌘ Barriers encountered when diagnosing patients with rare conditions

DIAGNOSTIC AND DISEASE KNOWLEDGE

- Lack of diagnostic tools or guidelines
- Disease mimics other diseases/process of elimination
- Not enough training on rare diseases
- Lack of information about rare diseases in your trusted medical sources
- Competing clinical priorities that delay further workup to diagnose properly

TESTING & COVERAGE CONCERNS

- Lack of access to genetic testing
- Lack of access to other diagnostic procedures or tests
- Out of pocket cost of testing for the patient
- Coverage denials for advanced or genetic testing

REFERRALS

- Limited access to specialists with rare disease expertise
- Uncertainty about when to escalate to advanced testing or referral
- Delays in referral to appropriate specialists
- Patients not following through on referrals due to logistics (travel) or other burdens

⌘ Typical information journey when investigating a suspected rare/hard-to-diagnose condition
(Ranked order, with 1 as the "first stop" and 5 as the "last stop")

- Professional peer-reviewed databases (UpToDate, Medline, etc.)
- Specialized rare disease registries (Orphanet, OMIM, etc.)
- Direct consultation with a specialist/sub-specialist
- Clinical AI or decision-support software
- Professional medical social media/crowdsourcing

TREATMENT JOURNEY & PATIENT INTERACTION

Share of decision-making with rare disease patients

- **Provider-Directed:** I provide the clinical recommendation and the patient usually follows my lead
- **Provider-Guided:** I make a recommendation, but it is heavily shaped by the patient's specific goals or preferences
- **Shared Partnership:** The patient and I weigh clinical options, goals and preferences as equal partners
- **Patient-Guided:** The patient comes with specific preferences; my role is to ensure the preferred treatment is clinically appropriate and effective

Type of primary practice

- Solo Practice
- Group Practice — Single or Multi-Specialty
- Community Hospital/Health-System
- Long Term Care Hospital/Health-System
- University/Teaching Hospital/Health-System
- Military/VA/Prison Hospital/Health-System
- Ambulatory Surgical Center
- Urgent Care Facility
- Telehealth Company



HCP Study (continued)

Challenges encountered most frequently when initiating or changing treatment (last 12 months)

FINANCIAL/ACCESS

- ♦ **Insurance/Prior Authorization:** Coverage denials or “step therapy” requirements
- ♦ **Patient Affordability:** Patient reported they couldn’t afford the out-of-pocket costs
- ♦ **Specialty Pharmacy Issues:** Lack of coverage or delays from specialty providers
- ♦ **Market Availability:** Medication was on shortage or discontinued by the manufacturer

CLINICAL/EFFICACY

- ♦ **Sub-optimal Efficacy:** The treatment was not meeting clinical endpoints or symptom control goals
- ♦ **Tolerability/Side Effects:** The patient experienced adverse events that outweighed the clinical benefit
- ♦ **Newer Clinical Data:** A more effective or safer “next-generation” treatment became available

PATIENT-LED/ADHERENCE

- ♦ **Treatment Burden:** The administration (e.g., frequent infusions, complex dosing) was too difficult for the patient’s lifestyle
- ♦ **Patient Request:** The patient/caregiver refused or requested a change based on their own research or preference
- ♦ **Loss to Follow-up:** The patient stopped coming to appointments or responding to treatment plans

💰 Usefulness of treatment/diagnosis aids

- ♦ **Direct MSL Access:** One-to-one scientific discussion with Medical Science Liaison
- ♦ **Medical Affairs Portals:** Pharma-provided
- ♦ **KOL Insights:** Expert-led clinical or treatment information
- ♦ **Payer Navigation Support:** Guidance to navigate prior authorizations/appeals
- ♦ **Reimbursement Assistance:** Information on patient assistance programs or out-of-pocket cost support

💰 Sources of evidence that most heavily influence decision to prescribe off-label (in the absence of FDA-approved therapies for the condition)
(Ranked on a scale of 1 to 6)

- ♦ **Clinical Logic:** Theoretical plausibility based on the drug’s Mechanism of Action
- ♦ **Expert Network Input:** Anecdotal success shared via professional platforms (e.g., Sermo, Doximity)
- ♦ **Institutional Protocols:** Treatment algorithms from a recognized Center of Excellence
- ♦ **Patient-Led Data:** Patient-reported outcomes or registry data provided by advocacy groups
- ♦ **Peer-Reviewed Data:** Small-scale RCTs or published case series
- ♦ **Professional Consensus:** Formal society guidelines or expert consensus statements

💰 Usefulness of patient educational resources

- ♦ Access to a nurse coordinator
- ♦ Condition-related patient/caregiver educational materials that are easy to understand
- ♦ Discussion guides for patients
- ♦ Information from patient advocates sharing their own experiences
- ♦ Information about patient support groups or online communities to share with patients
- ♦ Symptom tracking apps for patients

💰 Frequency patients bring health information found online/via AI to appointments

💰 Frequency that out-of-pocket cost concerns for patient influences choice of therapy — Always, Most of the time, Some of the time, Rarely, or Never

HCP Study (continued)

HEALTH INFORMATION NEEDS & RESOURCES

Useful sources for gathering information about rare/
hard-to-diagnose conditions
(1-5 scale)

MEDICAL WEBSITES

- ♦ Advocacy websites (e.g. Muscular Dystrophy Association, Platelet Disorder Support Association)
- ♦ Disease-Specific Websites (Professional)
- ♦ Government websites (e.g. CDC, NIH, clinicaltrials.gov)
- ♦ Medical Journals: Print/Websites
- ♦ Medical Society Websites
- ♦ Pharmaceutical company websites
- ♦ Websites with information about multiple rare diseases (e.g. NORD, CheckRare, etc.)

ONLINE TOOLS & RESOURCES

- ♦ Clinical AI Search Tools (e.g. OpenEvidence, UpToDateAI, etc.)
- ♦ EHR/EMR Platforms
- ♦ Mobile Apps: Diagnostic Tools
- ♦ Professional Portals: Free to Use (e.g., Medscape, MedPage Today, etc.)
- ♦ Professional Portals: Subscription-Based (e.g., UpToDate, ClinicalKey, etc.)

SOCIAL MEDIA, VIDEO & PODCASTS

- ♦ Key Opinion Leaders (KOLs)
- ♦ Healthcare Influencers (Online Medical Experts)
- ♦ Podcasts & Audio Education
- ♦ Social Media: Private MD Groups (e.g., Doximity, Sermo, Reddit, etc.)
- ♦ Webcasts/Webinars

CONFERENCES, PEERS & PHARMA

- ♦ Advocacy Summits: Joint Doctor/Patient Research Meetings
- ♦ Colleagues and Peers
- ♦ Grand Rounds/Speaker Programs
- ♦ Medical Affairs Platform Provided by a Pharmaceutical Manufacturer
- ♦ Medical Science Liaisons (MSLs)
- ♦ Multi-Disciplinary Case Reviews
- ♦ Pharmaceutical Sales Reps
- ♦ Rare Disease Conferences/Meetings (In-Person & Virtual)



Information missing/needed in better quality by
patients during their journey, by stage

BEFORE BEING DIAGNOSED

- ♦ Finding a specialist
- ♦ Identifying symptoms of the disease
- ♦ Understanding lab tests

AT THE TIME OF DIAGNOSIS

- ♦ How to explain diagnosis to loved ones
- ♦ Impact on life expectancy
- ♦ Managing daily life
- ♦ Typical course of the disease (prognosis)

WHEN DECIDING ON A TREATMENT

- ♦ Cost of treatment
- ♦ Dealing with insurance companies
- ♦ Programs to help pay for treatment
- ♦ Side effects and how to manage them

WHEN MANAGING DAILY LIFE

- ♦ Advice from other patients
- ♦ Dietary or nutritional information
- ♦ Mental health support

WHEN SYMPTOMS CHANGED OR TREATMENTS STOPPED WORKING

- ♦ Clinical trial eligibility
- ♦ New treatment options
- ♦ Understanding disease progression

💰 Resources relied on for treatment guidance when
managing a rare condition
(1-5 scale of importance)

- ♦ Published Guidelines (e.g., NCCN, AHA)
- ♦ Direct communication with a known KOL
- ♦ Consensus with local multidisciplinary peers

HCP Study (continued)

💰 Content formats most likely to turn to for information on rare conditions

QUICK REFERENCE (< 5 MIN)

- ♦ **Clinical Summaries:** Bulleted trial data or guideline updates
- ♦ **Visual Abstracts:** Infographics of study results
- ♦ **Short Video:** Expert “clinical pearls” or tech tips
- ♦ **Diagnostic Tools:** Digital checklists or “rule-in” criteria

DEEP DIVE (15+ MIN)

- ♦ **On-Demand Webcasts:** Full presentations from medical congresses
- ♦ **White Papers:** Detailed peer-reviewed monographs
- ♦ **Long-Form Audio:** Clinical podcasts or case-study interviews
- ♦ **Patient Simulations:** Interactive “walk-throughs” of a diagnosis

EXPERT & PEER INTERACTION

- ♦ **KOL Commentary:** Brief video editorials from top experts
- ♦ **Case Roundtables:** Videos of multidisciplinary teams discussing a case
- ♦ **Medical Portals:** Secure sites for MSL-provided high-level data

💰 Best specific platforms or websites for researching rare/hard-to-diagnose conditions

CLINICAL REFERENCE & DECISION SUPPORT

- ♦ Epocrates
- ♦ Healthgrades
- ♦ OpenEvidence
- ♦ UpToDate

MEDICAL LITERATURE & RARE DISEASE REGISTRIES

- ♦ Clinicaltrials.gov
- ♦ PubMed / MedLine (NLM)
- ♦ NIH/GARD (Genetic and Rare Diseases Information Center)
- ♦ NORD (National Organization for Rare Disorders)
- ♦ OMIM (Johns Hopkins University)
- ♦ Orphanet

PROFESSIONAL NEWS & AGGREGATORS

- ♦ CheckRare
- ♦ HCP Live
- ♦ Healio
- ♦ Medpage Today

- ♦ Medscape
- ♦ Rare Disease Advisor
- ♦ Rare Disease Network

PROFESSIONAL NETWORKING & PEER DISCUSSION

- ♦ Doximity
- ♦ PeerDirect
- ♦ Sermo

PROFESSIONAL USE OF AI

💰 Types of AI-powered activities used in practice

- ♦ **Admin/Operations:** Scheduling, billing, or finding diagnosis codes
- ♦ **Clinical Support:** Interpreting labs/scans or researching treatments/conditions
- ♦ **Documentation:** Charting, transcribing notes, or summarizing patient histories
- ♦ **Patient Interaction:** Drafting replies to messages or routine reminders
- ♦ **Prescribing:** Managing refills, drug interactions, or prior authorizations

💰 Primary concerns regarding AI use in practice

- ♦ **Clinical Risk:** Inaccuracies, “hallucinations,” or bias
- ♦ **Data Security:** Patient privacy and data ingestion
- ♦ **Operational:** Cost, implementation time, or lack of algorithmic transparency
- ♦ **Professional Risk:** Malpractice liability or loss of clinical nuance/skills
- ♦ I have no concerns

💰 Extent to which patients’ use of AI-driven tools influences their expectations about potential treatments or cures

- ♦ **Minimal:** Patients remain grounded and understand the experimental nature of the data
- ♦ **Moderate:** It leads to more questions but is generally manageable with a conversation
- ♦ **Significant:** It often creates ‘false hope’ for treatments that are not yet clinically viable or available
- ♦ **Critical:** It frequently leads to friction in the doctor-patient relationship when the AI’s ‘hopeful’ outlook conflicts with clinical reality

💰 Frequency patients bring health information found online/via AI to appointments

HCP Study (continued)



Attitudes about AI

- ♦ AI has made it easier to find information on rare or hard-to-diagnose conditions
- ♦ AI significantly improves my diagnostic accuracy for rare or hard-to-diagnose conditions
- ♦ Diagnostic AI has made it easier to determine which tests to use for diagnosis of rare or hard-to-diagnose conditions
- ♦ Patient use of AI fosters more productive clinical conversations around rare or hard-to-diagnose conditions

PROFESSIONAL USE OF SOCIAL MEDIA

Impact of social media Subject Matter Experts (SMEs) on clinical decision-making

- ♦ **Minimal Impact:** I find it interesting but rely almost exclusively on formal peer-reviewed journals for treatment decisions
- ♦ **Informational Impact:** It alerts me to new data or emerging trends, which I then verify through official clinical databases (e.g., PubMed, UpToDate)
- ♦ **Significant Impact:** I highly value their real-world clinical experience, especially for rare conditions where formal trial data may be limited or unavailable

💡 Usage & Frequency of the following to gather diagnostic advice for difficult cases

TYPES

- ♦ Professional digital communities (e.g., Sermo, Doximity)
- ♦ Closed healthcare provider groups (Reddit, Facebook, LinkedIn)
- ♦ Personal consultations with other healthcare providers

FREQUENCY

- ♦ At least once a week
- ♦ At least once a month
- ♦ Every 2-3 months
- ♦ Once or twice a year
- ♦ Never

Patient & Caregiver Study

This section includes all content asked in the Patient & Caregiver Study.

Notes: Topics and response options that were asked to Patients () and/or Caregivers () are indicated by their corresponding icon. Topics with no icon were asked in both the HCP and Patient & Caregiver studies, but details in this section reflect how questions were asked to patients and/or caregivers.

BASIC DEMOGRAPHICS

Age & Generation

Gender

Ethnicity

Hispanic/Latino/Spanish

EXPANDED RESPONDENT DEMOGRAPHICS

Geographical information

Language spoken at home — only/mostly Spanish, Spanish/English equally, only/mostly English

Marital status

Highest level of education completed

Employment status

Annual household income

Number of adults (& gender), children (& ages) in household

Parent to a child under 18

PATIENT PROFILE (CONDITION, HEALTH, ATTITUDES)

Current stage of patient's condition journey

- ◆ Recently diagnosed (within the last 6 months)
- ◆ Actively managing the disease and/or symptoms
- ◆ Currently evaluating or switching to a new treatment
- ◆ Currently experiencing a change or worsening of symptoms
- ◆ No longer experiencing symptoms or condition is stable

Current health of patient — excellent, very good, good, fair, or poor

Severity of patient's condition symptoms — very severe, severe, moderate, mild, or not noticeable

Impact of condition on patient's daily activities

PHYSICAL IMPACT

- ◆ Unable to climb steps
- ◆ Unable to lift 20 lbs from the floor
- ◆ Unable to walk without assistance
- ◆ Sensory impairment (e.g., vision, hearing)
- ◆ Speech impairment

WORK & COMMUTING

- ◆ Unable to drive a vehicle
- ◆ Physical barriers for using public transit
- ◆ Fatigue limiting tasks or activities
- ◆ Pain limiting tasks or activities
- ◆ Difficulty concentrating or remembering things
- ◆ Inability to work as much as desired

SOCIAL LIFE & INDEPENDENCE

- ◆ Depression
- ◆ Inability to socialize as much as desired
- ◆ Accessibility issues in home
- ◆ Require personal care assistance
- ◆ Require assistance with chores

Most important goals in treating patient's condition

- ◆ Being an active participant in family/children's lives
- ◆ Resuming normal social life with friends, hobbies, etc.
- ◆ Getting career/schooling back on track
- ◆ Being independent/able to take care of themselves
- ◆ Improving financial situation
- ◆ Not feeling like a burden to their family and friends
- ◆ Improving energy levels and reducing pain and/or fatigue
- ◆ Preventing condition from getting worse or slowing it down
- ◆ Improving mental focus and clarity
- ◆ Reducing need for ER visits or hospital stays
- ◆ Having more time for my own self-care, family, and social life —  CAREGIVER ONLY

Patient & Caregiver Study (continued)

PATIENT-SPECIFIC

Personal attitudes
(1-5 scale of agreement)

- ♦ Other patients come to me for advice about healthcare and medications
- ♦ I often feel anxious about how my condition affects my health or will affect it in the future
- ♦ I feel I have control over my health and quality of life

Received assistance from a caregiver related to rare condition(s) in the past 3 months

Provide caregiver support to a friend/family member for their health condition

HOME ASSISTANCE & CAREGIVING

Relation to caregiver (i.e. person primarily assisting with care)

- ♦ Spouse/partner
- ♦ Parent/guardian
- ♦ Grandparent —  CAREGIVER ONLY
- ♦ Child
- ♦ Sibling
- ♦ Grandchild —  CAREGIVER ONLY
- ♦ Another type of relative
- ♦ A friend
- ♦ Professional caregiver (not a friend or relative) —  PATIENT ONLY

Patient lives in same household as caregiver

Caregiver activities performed for patient

- ♦ Administer or monitor medications
- ♦ Buy medication or refill prescriptions
- ♦ Discuss conditions, medical choices, or treatments with doctor
- ♦ Make medical decisions for the patient
- ♦ Support the patient in making medical decisions
- ♦ Make medical appointments
- ♦ Provide transportation to appointments
- ♦ Research condition or treatment information
- ♦ Read information provided by the doctor and discuss it with the patient
- ♦ Advocate for the patient with insurers or health providers

Caregiver's level of involvement in patient's condition-related medical choices/treatment decisions
(1-5 scale)



CAREGIVER-SPECIFIC

Age of care recipient

Challenges faced as a caregiver

WORK-RELATED

- ♦ Reduced work hours, took time off or passed up promotion
- ♦ Lost job or resigned to focus on caregiving

LOGISTICAL

- ♦ Coordinating care across multiple health providers
- ♦ Dealing with insurance companies
- ♦ Arranging transportation to appointments

SUPPORT

- ♦ Finding caregiver communities online for information and support
- ♦ Finding accurate condition or treatment information
- ♦ Needing a break or help with daily care

PERSONAL

- ♦ Putting my own life goals or career on hold
- ♦ Meeting my own family or other responsibilities

Personal & caregiver attitudes

- ♦ I have adequate support from family, friends, or community in caring for my loved one
- ♦ I feel confident understanding and applying health information to care for my loved one
- ♦ I feel socially isolated due to my caregiving responsibilities
- ♦ I often feel overwhelmed and anxious with the responsibility of caring for my loved one
- ♦ I feel confident navigating the healthcare system and insurance for my loved one
- ♦ Other caregivers come to me for advice about healthcare and medications
- ♦ I often feel anxious about how my loved one's condition affects their health or will affect it in the future
- ♦ I feel I have control over my loved one's health and quality of life

Patient & Caregiver Study (continued)

SOCIAL DETERMINANTS OF HEALTH

Challenges related to *respondent's* personal health/ conditions in the last 12 months

- ♦ Lack of health insurance or inability to afford your healthcare or medicines
- ♦ Lack of pharmacies that carry or can order your medicines
- ♦ Lack of stable housing or frequent changes in your housing situation
- ♦ Difficulty affording food or getting healthy food
- ♦ Difficulty understanding medical information or making decisions about your health
- ♦ Inability to access transportation for medical appointments
- ♦ Unsafe neighborhood or living environment
- ♦ Lack of friends and family to assist with healthcare needs
- ♦ Difficulty maintaining employment or lack of access to educational or job opportunities
- ♦ Discrimination based on your race, ethnicity, gender, or sexual orientation
- ♦ Language barriers at medical appointments or when reviewing medical information

DIAGNOSIS JOURNEY

Time from appearance of symptoms to diagnosis with rare condition

Patient ever received a wrong diagnosis

 Time from misdiagnosis to correct one

How found doctor who correctly diagnosed rare condition

- ♦ Recommended by primary care provider
- ♦ Recommended by another doctor or specialist
- ♦ Recommended by another patient or caregiver (in person or online)
- ♦ Recommended by a friend or family member
- ♦ Searching insurance provider network
- ♦ Looked on a website for patients with this condition
- ♦ General online search (e.g. Google, Bing)
- ♦ AI platforms or tools (e.g. ChatGPT, Claude)
- ♦ Don't know/not sure



 Tests needed before receiving correct diagnosis

- ♦ Screening tests for pregnant mothers or newborn babies
- ♦ Imaging such as ultrasound, x-rays, MRI, CT scans, etc.
- ♦ Blood tests (other than genetic or DNA testing)
- ♦ Physical exams
- ♦ Genetic or DNA tests
- ♦ Tests of your body or brain function (e.g. neurological, cognitive, or mobility tests)
- ♦ Biopsies

TREATMENT JOURNEY & HCP INTERACTION

 Treatment status — currently treated, previously treated, or untreated

 Current treatments used for rare condition

- ♦ Non-prescription drugs (over-the-counter)
- ♦ Prescription drugs
- ♦ Steroids
- ♦ Infusions or injections
- ♦ Blood products/transfusions
- ♦ Chemotherapy/Radiation/Targeted therapy
- ♦ Immunotherapy
- ♦ Gene therapy
- ♦ Oxygen therapy/supplemental oxygen
- ♦ Surgery/Transplant
- ♦ Special diets (e.g. keto, gluten-free, etc.)
- ♦ Herbal supplements or vitamins
- ♦ Physical therapy
- ♦ Medical marijuana
- ♦ Counseling or behavioral therapy

 Time from diagnosis to first starting treatment

Patient & Caregiver Study (continued)

Share of decision-making with HCP seen most often for managing condition

- ♦ The doctor makes most of the decisions; I/we follow their lead
- ♦ A partnership; we make decisions together
- ♦ I/we make most of the decisions; the doctor follows our lead
- ♦ I'm not involved in medical decision-making

 Attitudes about the HCP primarily treating the condition

- ♦ I trust the doctor's expertise in managing the condition
- ♦ The doctor communicates in a way I can easily understand
- ♦ I feel confident discussing information I found online with the doctor
- ♦ I feel frustrated by having to do research to educate the doctor on treatment options

Healthcare facilities/locations used for care

- ♦ Hospital
- ♦ Center of Excellence
- ♦ Doctor's office
- ♦ Infusion center
- ♦ Local clinic or behavioral/physical therapy office
- ♦ Home visits from a nurse or therapist
- ♦ Virtual visits on a telemedicine app

 Has patient ever stopped or changed treatment for rare condition

 Reasons for stopping/changing treatment

FINANCIAL/INSURANCE

- ♦ Insurance would no longer cover it
- ♦ Couldn't afford the treatment anymore (regardless of insurance coverage)
- ♦ Didn't have access to a specialty pharmacy
- ♦ No longer available on the market
- ♦ Lost insurance coverage

CLINICAL/LOGISTICS

- ♦ It wasn't working/not effective
- ♦ Too many side effects
- ♦ Doctor recommended stopping or changing treatment
- ♦ Condition improved or symptoms got better
- ♦ Major life changes (moved away, lost or changed a job, pregnancy, etc.)

PERSONAL REASONS

- ♦ Too difficult to manage all the paperwork/forms/phone calls
- ♦ Travel to appointments was too burdensome
- ♦ Treatment was too difficult to self-administer
- ♦ Got tired of doing the treatment
- ♦ Another reason (specify)

Reasons patient's doctor changed/stopped treatment

- ♦ A newer treatment became available
- ♦ Interacted/not allowed with another medication or treatment being used
- ♦ End of participation in a clinical trial
- ♦ Insurance or reimbursement issues
- ♦ Difficulty with prior authorization or paperwork
- ♦ Medication unavailable or too difficult to obtain



TREATMENT IMPACT & COST

Impact of current treatment regimen on patient's daily life
(1-5 scale, "Not at all" to "Extremely" burdensome)

Attitudes about current treatment regimen

- ♦ It makes the [condition symptoms] better and/or easier to manage
- ♦ Being on this treatment gives hope for [the patient's] future
- ♦ The side effects of this treatment makes [the patient] feel worse in the short term

Patient's insurance type

- ♦ Employer or union provided
- ♦ Medicaid
- ♦ Medicare
- ♦ Veteran/retired military (VA)
- ♦ Government employee or active military
- ♦ Private or self-paid plan
- ♦ Health insurance marketplace (ACA)
- ♦ Other
- ♦ No insurance

Patient & Caregiver Study (continued)

How paid for prescriptions in last 12 months

- ♦ Drug company assistance programs
- ♦ Government platforms
- ♦ Third-party discount programs
- ♦ Drug company copay cards or coupons
- ♦ Income-based assistance or charity
- ♦ Store discount programs

Actions taken to help pay for patient's treatment (last 12 months)

PERSONAL FINANCE

- ♦ Dip into savings or retirement funds
- ♦ Take on new debt (refinance home, credit card debt, etc.)

LIFESTYLE CHANGES

- ♦ Work longer hours or take an extra job
- ♦ Change your living arrangements to save money
- ♦ Cut back on social activities to save money
- ♦ Cut back on essentials (e.g., groceries, utilities)
- ♦ Skip or delay other healthcare

OUTSIDE ASSISTANCE

- ♦ Apply for patient assistance or charity programs
- ♦ Crowdfunding (e.g., start a GoFundMe or requested financial help from others)

Impact of cost concerns on **respondent's** stress level (1-5 scale, "Not at all" to "Very" stressful)

HEALTH INFORMATION NEEDS & RESOURCES

Sources used & Trust in them for gathering information about patient's rare condition(s) (1-5 scale, "Do not trust at all" to "Trust completely")

HEALTH & MEDICAL WEBSITES

- ♦ General health sites (WebMD, Mayo Clinic, etc.)
- ♦ Government health sites (NIH, clinicaltrials.gov, etc.)
- ♦ Rare disease organization sites (NORD, MDA, etc.)
- ♦ Medical journals or healthcare professional sites (PubMed, Medscape, etc.)

SEARCH & AI

- ♦ Search engines (Google, Bing, etc.)
- ♦ AI tools (Gemini, ChatGPT, etc.)

POINT OF CARE

- ♦ Patient portal (MyChart, Healow, etc.)
- ♦ Doctor-provided materials (printed or digital)

SOCIAL MEDIA & PEER COMMUNITIES

- ♦ Specialized social groups (Private Facebook Groups, Subreddits, etc.)
- ♦ Condition-specific online communities (Inspire, MyHealthTeams, etc.)
- ♦ Health influencers or patient advocates

VIDEO & PODCASTS

- ♦ Video platforms (YouTube, TikTok, etc.)
- ♦ Health-related podcasts (doctor or patient-hosted)

 Stage(s) in patient's journey where information felt lacking (in quality or quantity) to make a confident decision

- ♦ Before being diagnosed
- ♦ At the time of diagnosis
- ♦ When deciding on a treatment
- ♦ Managing daily life
- ♦ When symptoms changed or treatment stopped working

Types of information that was lacking (by stage)

BEFORE DIAGNOSIS

- ♦ Identifying symptoms of the disease
- ♦ Finding a specialist
- ♦ Understanding lab tests

AT THE TIME OF DIAGNOSIS

- ♦ Typical course of the disease (prognosis)
- ♦ Impact on life expectancy
- ♦ How to explain diagnosis to loved ones
- ♦ Managing daily life

WHEN DECIDING TREATMENT

- ♦ Side effects and how to manage them
- ♦ Cost of treatment
- ♦ Dealing with insurance companies
- ♦ Programs to help pay for treatment

WHEN MANAGING DAILY LIFE

- ♦ Mental health support
- ♦ Advice from other patients
- ♦ Dietary or nutritional information

WHEN SYMPTOMS CHANGED OR TREATMENTS STOPPED WORKING

- ♦ Understanding disease progression
- ♦ New treatment options
- ♦ Clinical trial eligibility

Patient & Caregiver Study (continued)

 Reasons for using trusted sources (by type)

HEALTH & MEDICAL WEBSITES

- ♦ I only trust information that has been vetted by the FDA or medical boards
- ♦ I use them to find cost-assistance or clinical trial information
- ♦ Data privacy
- ♦ Educate myself with the most recent medical breakthroughs

SEARCH & AI

- ♦ I can turn complex medical reports into easy-to-read summaries
- ♦ I can ask questions without being judged by a doctor or nurse
- ♦ I can ask questions 24/7

SOCIAL MEDIA & PEER COMMUNITIES

- ♦ I want to hear from someone who lives with [the rare condition] symptoms every day
- ♦ I feel more comfortable asking sensitive questions here than a doctor's office
- ♦ I get advice on living with [patient's] disease that I can't get anywhere else
- ♦ I use them to see if others have experienced the same side effects from treatment

VIDEO & PODCASTS

- ♦ I need to see a physical demonstration
- ♦ It allows me to hear from medical experts I would never be able to see in person
- ♦ Seeing/hearing other [patients/caregivers] makes me feel less isolated



ONLINE HEALTH & ADVERTISING

Attitudes about online health/condition resources

- ♦ I am willing to share [the patient's] health data online if I believe it will help researchers find better treatments
- ♦ I refer other [patients/caregivers] to websites I find helpful
- ♦ I typically conduct research online prior to a doctor's appointment
- ♦ I trust the medical information other [patients/caregivers] share on social media
- ♦ Information I find online influences the decisions I make about treating and/or managing [the condition]
- ♦ Finding an online community for [the rare condition] has made me feel less alone in my journey
- ♦ Sometimes looking at condition-specific information online makes me feel overwhelmed or anxious rather than empowered
- ♦ I feel confident in my ability to distinguish between high-quality medical evidence and "fake news" or unverified claims online

Percent of internet time spent consuming health- or medical-related information

Online health-related activities performed on computer/mobile, by type (in the last 12 months)

RESEARCH & VALIDATION

- ♦ Looked for information on a condition, symptoms, or medications
- ♦ Sought a second opinion or researched treatment options after a doctor's visit
- ♦ Used an AI tool (e.g., Gemini, ChatGPT) to research health info

SOCIAL & PEER SUPPORT

- ♦ Read about others' experiences or shared your own on social media
- ♦ Followed health-related posts by advocates, influencers, or medical professionals
- ♦ Sought advice, encouragement, or support from other patients or caregivers

CARE COORDINATION & NAVIGATION

- ♦ Looked for a doctor, pharmacy, or treatment center (including reading reviews)
- ♦ Compared medication prices or purchased/refilled a prescription online
- ♦ Scanned a QR code to access health or treatment information

EDUCATION & MEDIA

- ♦ Watched videos to learn about symptoms, conditions, or treatments
- ♦ Listened to health-related or medical podcasts

Patient & Caregiver Study (continued)

Where seen/heard healthcare advertising in the past 12 months

- ◆ Direct mail to your home
- ◆ Doctor's office
- ◆ Pharmacy
- ◆ Broadcast or cable television
- ◆ Television streaming service/app (e.g., Amazon Freevee, Paramount+, Tubi)
- ◆ Internet
- ◆ Mobile device (cell phone/smartphone or tablet)
- ◆ Social media
- ◆ Radio
- ◆ Smart speaker (e.g., Amazon Echo, Google Nest)
- ◆ Podcast

Actions taken as a result of the healthcare advertising

- ◆ Asked your doctor for a product sample of a prescription drug
- ◆ Asked your doctor to prescribe a specific drug
- ◆ Made an appointment to see a doctor
- ◆ Discussed an ad with your doctor
- ◆ Consulted a pharmacist
- ◆ Called a toll-free number to get additional information
- ◆ Discussed an ad with a friend or relative
- ◆ Signed up for a mail/email list to receive more information
- ◆ Conducted an online search
- ◆ Visited a pharmaceutical company's website
- ◆ Visited some other health website
- ◆ Watched a video online
- ◆ Refilled a prescription
- ◆ Switched to a different brand
- ◆ Took medication
- ◆ Used a coupon

Attitudes about pharma & advertising

- ◆ I am more likely to trust a pharma company if their ads highlight patient support and assistance programs
- ◆ I feel that pharma ads accurately reflect the reality of living with [patient's] condition
- ◆ Pharma ads help me better understand the symptoms and progression of [patient's] condition
- ◆ I trust pharmaceutical companies that advertise the medications [patient takes]

USE OF AI FOR HEALTH

 AI platforms used for health purposes

- ◆ ChatGPT
- ◆ Claude
- ◆ DALL-E
- ◆ Google Gemini
- ◆ Microsoft Copilot
- ◆ Perplexity

 Types of AI tools used in managing patient's health

- ◆ Symptom checkers (e.g. WebMD, Ubie, Isabel, etc.)
- ◆ Online chatbot to answer questions about medications, symptoms, or a specific medical condition
- ◆ Fitness tracker data analyzers (e.g. Athlytic, FitnessAI, etc.)

 Important features of AI tool when using for information about rare condition or treatment

- ◆ Reducing complex medical terms to just the essentials you need to know
- ◆ Summarizing long medication guides into quick-read summaries and lists
- ◆ Ability to ask follow-up questions via interactive tool or chatbot
- ◆ Personalized information to [patient's] age or other health conditions
- ◆ Personalized information to [patient's] specific lifestyle (how treatment impacts travel, work, etc.)
- ◆ Seeing which medical journal or advocacy groups the AI used to answer my questions
- ◆ Finding new clinical trials or specialists in my area

Attitudes about AI

- ◆ The AI-generated summaries or overviews in search results meet my needs when looking for health information related to [the patient's] condition
- ◆ I trust AI-generated recommendations as much as I trust advice from a doctor or other healthcare provider
- ◆ Using AI tools or summaries have helped me manage [the rare] condition more effectively

Patient & Caregiver Study (continued)



SOCIAL MEDIA

Impact of health influencers/patient advocates on social media (e.g., as a trusted source, reasons for trusting)

 Percent of internet time spent on social media

 Social media platforms used for any purpose

- ◆ Bluesky
- ◆ Discord
- ◆ Facebook
- ◆ Instagram
- ◆ LinkedIn
- ◆ Reddit
- ◆ Threads
- ◆ TikTok
- ◆ X (fka Twitter)

 Patient community websites used

- ◆ Ben's Friends
- ◆ Daily Strength
- ◆ Inspire
- ◆ Health Union
- ◆ Health Unlocked
- ◆ MyHealthTeams
- ◆ Patients Like Me
- ◆ Sharecare

MEDIA USAGE

Hours spent watching television on a typical weekday & weekend

Proportion of personal TV watching in a typical week, by viewing method

- ◆ Cable, satellite, or broadcast TV
- ◆ Smart TV/Internet-enabled TV
- ◆ Media streaming device (e.g., Amazon Fire TV Stick, Apple TV, Roku)
- ◆ On a laptop or mobile device using a browser or streaming app (e.g., Amazon Prime, Netflix, Peacock)

Hours spent listening to radio, streaming music, or podcasts on a typical day

Types of audio listened to

- ◆ Radio (traditional/terrestrial or internet radio stations)
- ◆ Streaming music through an online service or app (e.g., Spotify, Apple Music)
- ◆ Podcasts

Hours spent online on a typical day

Percent of internet time spent using a mobile device

Hours spent watching online video on a typical day

Time spent with media (in quintiles) for:

- ◆ Television - Linear TV (Cable, satellite, or broadcast)
- ◆ Television - Streaming TV
- ◆ Television (Any Platform NET)
- ◆ Audio (Radio, Streaming Music, or Podcasts)
- ◆ Internet on mobile device
- ◆ Internet on social media
- ◆ Internet (Any Device)
- ◆ Online video



OTHER SYNDICATED OFFERS:**Sources & Interactions Study (U.S.)**

Preferences and usage across 40+ media channels, type of information preferred by channel, patient engagement along with social media and other digital channel behaviors and motivations.

S&I: International

Essential insights into European and Asian healthcare professionals (alternating each year). Currently available for France, Germany, Italy, Spain, the United Kingdom, China, and Japan.

Ad Intelligence

The market's leading competitive ad intelligence platform, used by brands, agencies and media to understand advertising share of voice, competitive strategies and spend across media channels. Data is collected, classified and updated daily.

MARS Consumer Health Study

MARS is the industry's most comprehensive dataset of health consumers, connecting 100+ conditions with treatments, Rx brands, media usage, health behaviors, attitudes, and more. Developed from a nationally representative data set of 40K U.S. adults, M3 MI's annual bilingual survey provides robust samples sizes and coverage of low incidence conditions.

Doctors as Consumers

A dive into who physicians are as people, including their interests, values, and media used for personal reasons, covering much of the same content as our annual MARS Consumer Health Study.

Media Measurement Studies

Detailed reporting on physician exposure to content and advertising for 600+ websites/portals, 50+ apps, over 500 publications and print/online drug references, social media, and more, with specific use of measured properties by specialty to develop media plans across 33 HCP audiences.

Other HCP Specialties & Health Verticals

Media measurement and strategic omnichannel insights for 20+ medical/surgical physician specialties, and other health verticals including: dentistry, managed care, oncology nursing, PA/NP, and more. View the [full list](#) on our website.

Flexible Data Access

Our data is available to clients via multiple platforms and formats for maximum convenience and utility. Data delivery options include:

- Dashboards - Delivery dashboards for syndicated research data
- Online crosstab analytic tool via TelmarHelixa
- Customized Solutions - Don't see exactly what you need? Within any of our platforms and deliverables, we can help create custom filters or exports to fulfill client needs

If you have any questions or would like to learn more, visit our website or contact:

Client Services Hotline: ClientService@m3-mi.com

General Questions: info@m3-mi.com