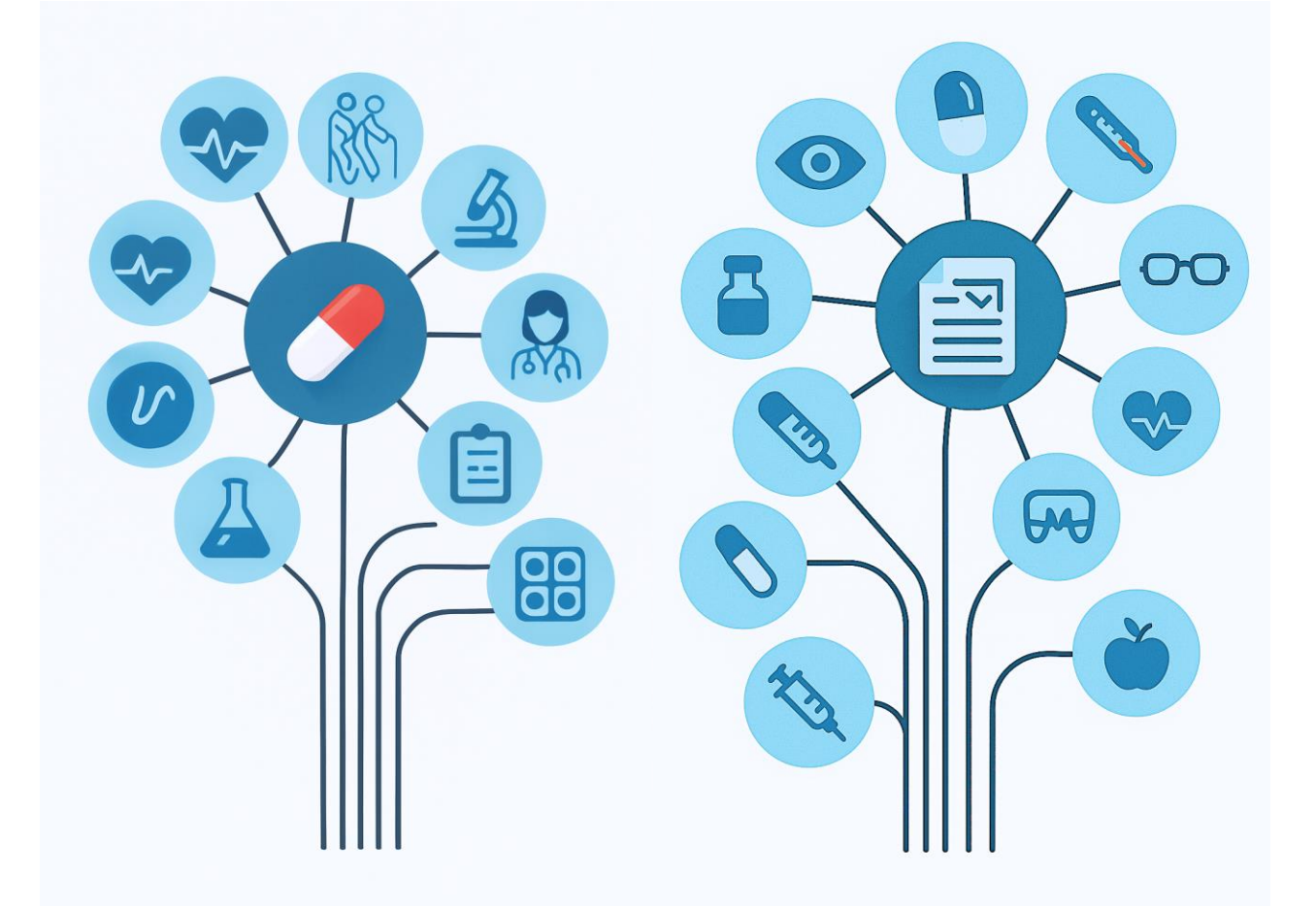


Empowering Caregivers & Researchers with MiraKare: A Platform for Real-World Evidence in Rare Disease Care

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ABSTRACT

Rare diseases are defined as conditions affecting fewer than 1 in 2,000 people in any WHO region (under 65 per 100,000 individuals). These include rare genetic disorders, cancers, infections, poisonings, immune-related conditions, idiopathic diseases, and other undiagnosed or unexplained conditions.

A major challenge in addressing rare diseases is the lack of comprehensive data. Caregivers, especially family members, face numerous unmet needs—ranging from delayed diagnoses and limited access to specialists, to difficulties in obtaining appropriate treatments. Even with a diagnosis, clinical care is often inadequate. The social and emotional toll on families adds to the long-standing public health burden.

To explore these issues, HITLAB conducted stakeholder interviews to better understand caregiver needs, data gaps, and system-level challenges. These insights are informing the development of MiraKare’s caregiver support platform, helping refine its design to improve outcomes for both patients and their caregivers.

OBJECTIVES

- Gather insights from leading rare disease organizations on key aspects of treatment and care—including data collection, analysis, and caregiver support.
- Specifically, understand current practices and challenges in real-world evidence (RWE) data collection from the perspectives of patients and caregivers.
- Explore how digital technologies can empower caregivers to contribute meaningful data and enhance research and care outcomes.

METHODOLOGY

- This qualitative study involved in-depth, one-on-one structured interviews with eight decision-makers from rare disease organizations, including researchers, caregiver groups, and providers.
- Each interview lasted 60–90 minutes and covered 21 core questions, with additional follow-ups based on the interviewee’s expertise.
- Topics included data collection and quality, advocacy, technology adoption, wearable integration, patient-caregiver interactions, and the potential benefits of MiraKare. Logos of participating organizations are shown below.
- The data was analyzed using methods of narrative, thematic and quantitative analysis, where applicable. Thematic analysis revealed key take-aways from the qualitative responses.

RESULTS

Current State of RWE Data Collection

- **Fragmented and Limited:** Most data collected today focuses on clinical measures or episodic surveys; daily lived experiences and caregiver observations are largely missing.
- **Minimal Caregiver-Centric Data:** Few systems capture caregiver stress, burden, or insights about subtle patient changes — despite caregivers being closest to daily realities.
- **Engagement Barriers:** Caregivers are overwhelmed; traditional data collection methods (long surveys, infrequent touchpoints) are burdensome and lead to low sustained participation.
- **Platform as Population Resource:** There is positive interest in using caregiver platforms as a foundation for cohort development, natural history studies, and real-world data (RWD) collection to support broader research efforts.

Measurement, Monitoring & Digital Enablement

- **Caregiver-Specific Metrics:** Stakeholders recommended incorporating validated tools to measure caregiver stress, workload, and emotional well-being, alongside disease-specific metrics for patients.
- **Medication Monitoring:** The need for features that track medication adherence in real-time—including time-of-day verification (e.g., whether morning doses are taken as scheduled)—was highlighted.
- **Video Upload Capability:** Families expressed interest in being able to upload videos (e.g., documenting symptoms or motor function), adding a valuable visual layer to patient records.
- **Correlating Data Sets:** The ability to cross-reference caregiver-entered data—such as food diaries, sleep patterns, or symptom logs—was seen as critical to improving clinical insights and personalized care.
- **Resource Accessibility:** Easy access to reliable, centralized information (e.g., via QR codes, direct links, or embedded tools) is essential to supporting both patients and caregivers.
- **Time-Saving Features:** Participants suggested automating repetitive data collection tasks and alerts (e.g., appointment reminders, medication schedules) to reduce caregiver burden.
- **Peer-to-Peer Communities:** A dedicated, secure space for both caregivers and patients to connect, share, and support one another was viewed as a vital emotional and informational support feature.

Gaps in Care and Systemic Challenges

- **Caregiver Burden:** Caregivers experience significant emotional, physical, and economic stress, often affecting productivity and quality of life.
- **Language and Cultural Barriers:** A lack of multilingual resources and culturally competent care hinders engagement, particularly in diverse populations.
- **Provider Constraints:** A decline in provider availability, combined with limited clinician time and understanding of rare diseases, compromises care quality.
- **Enrollment and Participation Challenges:** Families face hurdles in enrolling in research or registries due to time, understanding, or lack of outreach.
- **Fragmented Data Ecosystems:** Disconnected platforms and data silos prevent a comprehensive view of patient health and care coordination.
- **Ethical and Practical Use of AI:** While there is optimism around AI, concerns remain around responsible implementation, data privacy, and equitable access.

Quotes from Interviewees

"Rare disease organizations are diverse — one size does not fit all. Platforms must adapt to different diseases, family structures, and needs."

"Engage caregivers — they are the missing link in understanding the day-to-day lived experience that clinical trials miss."

"Families have a treasure trove of daily information — but today, most of it’s lost because there’s no easy way to capture it."

"Technology has transformed every part of our lives — except caregiving. It’s time we bridge that gap for research too."

Intended Outcomes and Strategic Focus Areas

- **Support for Patients and Families**
 - Deliver targeted support programs based on need and disease stage.
 - Enable peer support by connecting families undergoing similar experiences.
 - Promote regular pediatric screenings and early detection—especially given the high prevalence of rare diseases in children.
- **Advocacy and Equity**
 - Advocate at the federal, state, and local levels for better rare disease care and resources.
 - Promote health equity by addressing underrepresentation of marginalized populations.
 - Support collective advocacy by fostering disease coalitions.

Research and Clinical Trial Access

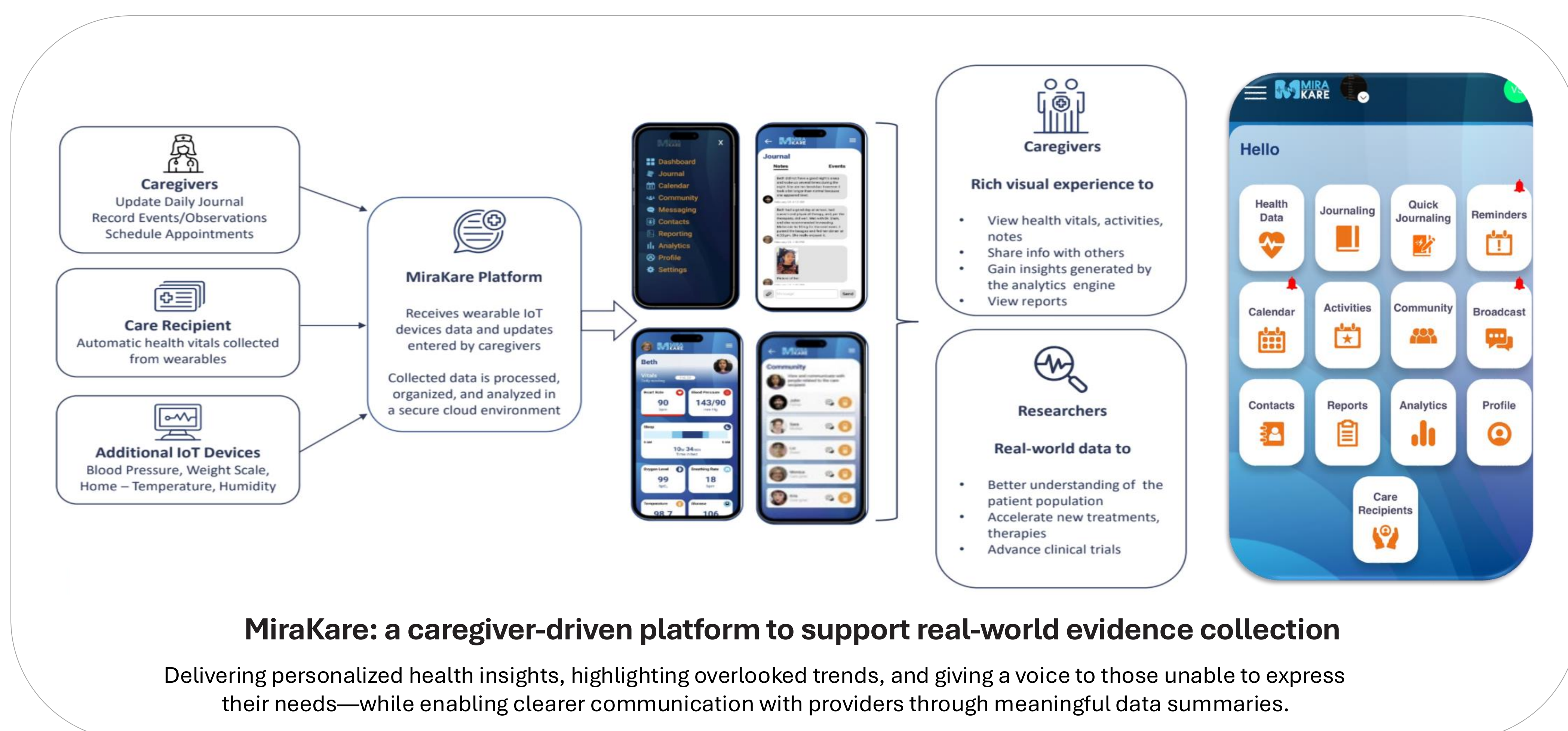
- Facilitate caregiver and patient enrollment in clinical trials and natural history studies.
- Encourage deeper caregiver engagement in research processes.
- Build strong networks between caregivers, researchers, and pharmaceutical companies.
- Develop clinical care center networks tied to major research institutions.

Toward a Comprehensive View of Care

- Set up robust patient registries that are interoperable and inclusive.
- Leverage AI to accelerate insights, lower costs, and create dynamic, adaptive care models.
- Address the fragmentation of data across systems to provide caregivers and clinicians with a unified, actionable picture of the patient’s health journey.

CONCLUSIONS

- Revealed critical caregiver needs across emotional, clinical, and logistical domains. Highlighted gaps in care, data collection, and access to trusted resources.
- Emphasized the importance of real-time insights and stress-reducing tools for caregivers. Identified strong demand for peer support, caregiver-focused metrics, and unified data platforms.
- MiraKare is actively incorporating these insights into its platform to deliver smarter support, enable connected caregiving communities, and drive better outcomes for both families and researchers.



MiraKare: a caregiver-driven platform to support real-world evidence collection

Delivering personalized health insights, highlighting overlooked trends, and giving a voice to those unable to express their needs—while enabling clearer communication with providers through meaningful data summaries.

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