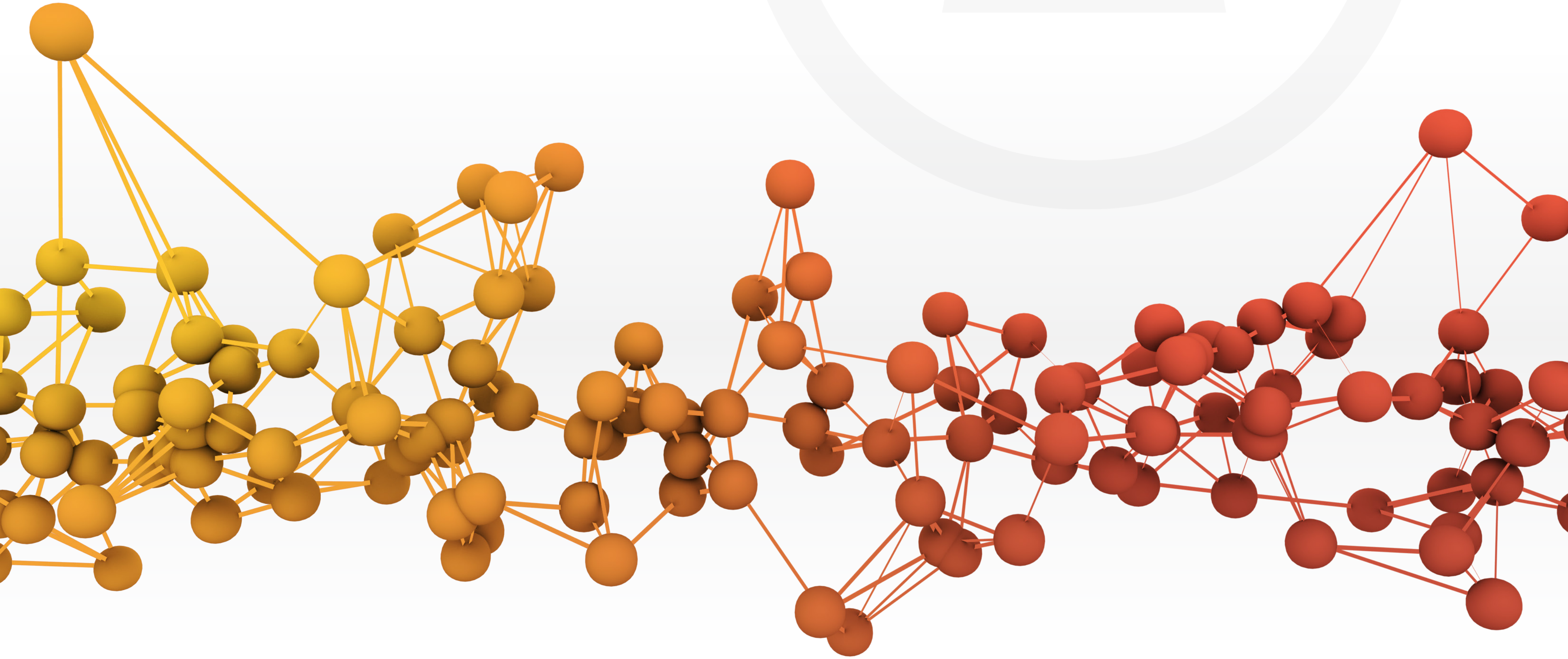
CMTC and Vascular Malformations

Community Voice Report

VOLUME 1, ISSUE 1 - MARCH 2020



DISEASE CATEGORY

SKIN
CIRCULATION/CARDIOVASCULAR

TREND Community™
Turning Anecdotes Into Evidence™

Capturing Patient Experience Data

The Problem

Capturing patient experience data and applying it to the drug development and regulatory process is often lengthy, laborious, and expensive. This is further complicated for rare diseases where affected patient populations are typically small, heterogeneous, and widely dispersed.

The Solution

TREND analyzes years of real-world experience data shared within social networks to gain valuable insights into the community's perspective on living with rare disease. These data are de-identified and summarized into a Community Voice Report, which can then lead to Data Explorations and Health Initiatives.

Community Voice Report

The Community Voice Report follows the FDA's patient input Guidelines for Patient-Focused Drug Development meetings and aims to quantify disease burden, disease management strategies, and possible unmet needs.

Data Explorations

TREND Data Explorations further analyze existing social data to uncover deeper insights. Unlike the breadth of a Community Voice Report, Data Explorations examine a single topic and reanalyze the surrounding posts, comments, and feedback to better understand the community's interest.

Health Initiatives

TREND Community Health Initiatives bring community members together on the TREND Community™ platform to collectively track specific data and explore potential solutions to address an identified unmet need.

> ***"The TREND report was invaluable in helping us define the questions and multiple-choice answers that should be included in our Externally Led Patient Focused Drug Development Meeting last June."***
>
> *—Kyle Bryant, FARA, rideATAXIA Founder/*
>
> *Program Director*

How It Works

All of our projects start with community engagement. Due to the unique difficulties of rare and chronic disease, many groups have created social networks online to support and validate each other. In these groups, community members are able to tell stories, promote advocacy, and share advice for managing symptoms, navigating the health care system, and living with a rare or chronic disease. We connect with individuals and leaders in these communities to learn about their illnesses, understand the current state of medical knowledge, and determine how our partnership can best address their unmet needs.

Once we have established a relationship with the community and gained consent, we download the deidentified data from these social media streams. These data are run through our analytics engine, named Krystie after the dear daughter of one of our community members. Krystie quickly analyzes vast amounts of data from years' worth of community discussion. Analysts manually code a sample of the data to validate the algorithms and train the engine to identify deeper insights (for example, to detect whether the speaker is a patient or a caregiver). This analysis culminates in a custom Community Voice Report.

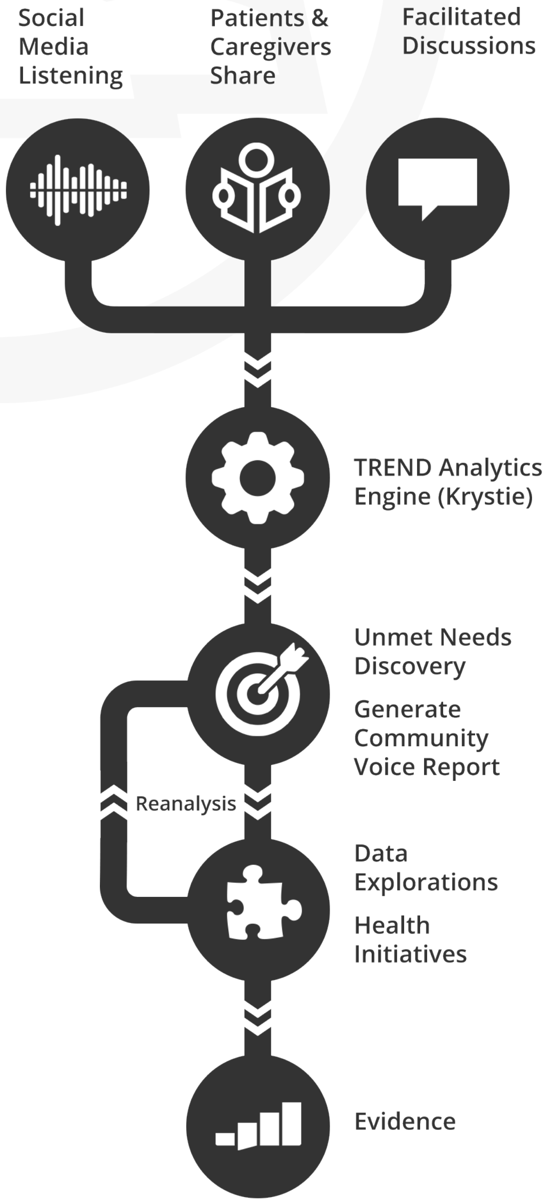
These Community Voice Reports are shared with the community free of charge, and our industry stakeholders have the opportunity to license the data for projects that address unmet needs and improve quality of life. These insights may be used to design Data Explorations or Health Initiatives.

Industry Stakeholders use TREND data to:

- Establish disease natural history
- Identify unmet therapeutic needs
- Understand quality of life issues
- Design better clinical trials
- Build a case for patient-centered regulatory approvals

Community Stakeholders use TREND data to:

- Inform medical and support teams
- Educate family members
- Catalyze research
- Spread awareness and advocacy



Analysis Background

Cutis Marmorata Telangiectatica Congenita (CMTC) is a birth defect that affects the skin and blood vessels. Symptoms include patches of marbled-looking skin (cutis marmorata), small widened blood vessels under the skin (telangiectasia), and varicose veins (phlebectasia).[1]

TREND analyzed a single Facebook group for this report.[2] This group was created by patients and caregivers as a place where members could share advice and lend support regarding CMTC and other vascular malformations. At the time of analysis, there were 799 members in the group.

> *"… she was almost one and a half years old before we knew what kind of deviation she had."*
>
> *— Community Member*

780
PEOPLE
PARTICIPATING

2,712
POSTS
SHARED

15,424
COMMENTS
ELICITED

FEB 2009 - JAN 2020
DATE RANGE

Disease Burden

CMTC has many physical symptoms. Some of the most frequently discussed terms were *skin colorations* (1087 all-time mentions/46 mentions in the last year), *vascular* (673/35), *pain* (883/28), *ulcer* (99/28), *cold body temperature* (262/14), *illness* (127/9), *swelling* (244/4), and *fever* (114/4). Skin colorations include all of the color terms that appeared to describe skin color because of vein anomalies. *Clots* (349) and *Glaucoma* (271) were not discussed in the last year but were mentioned frequently before then.

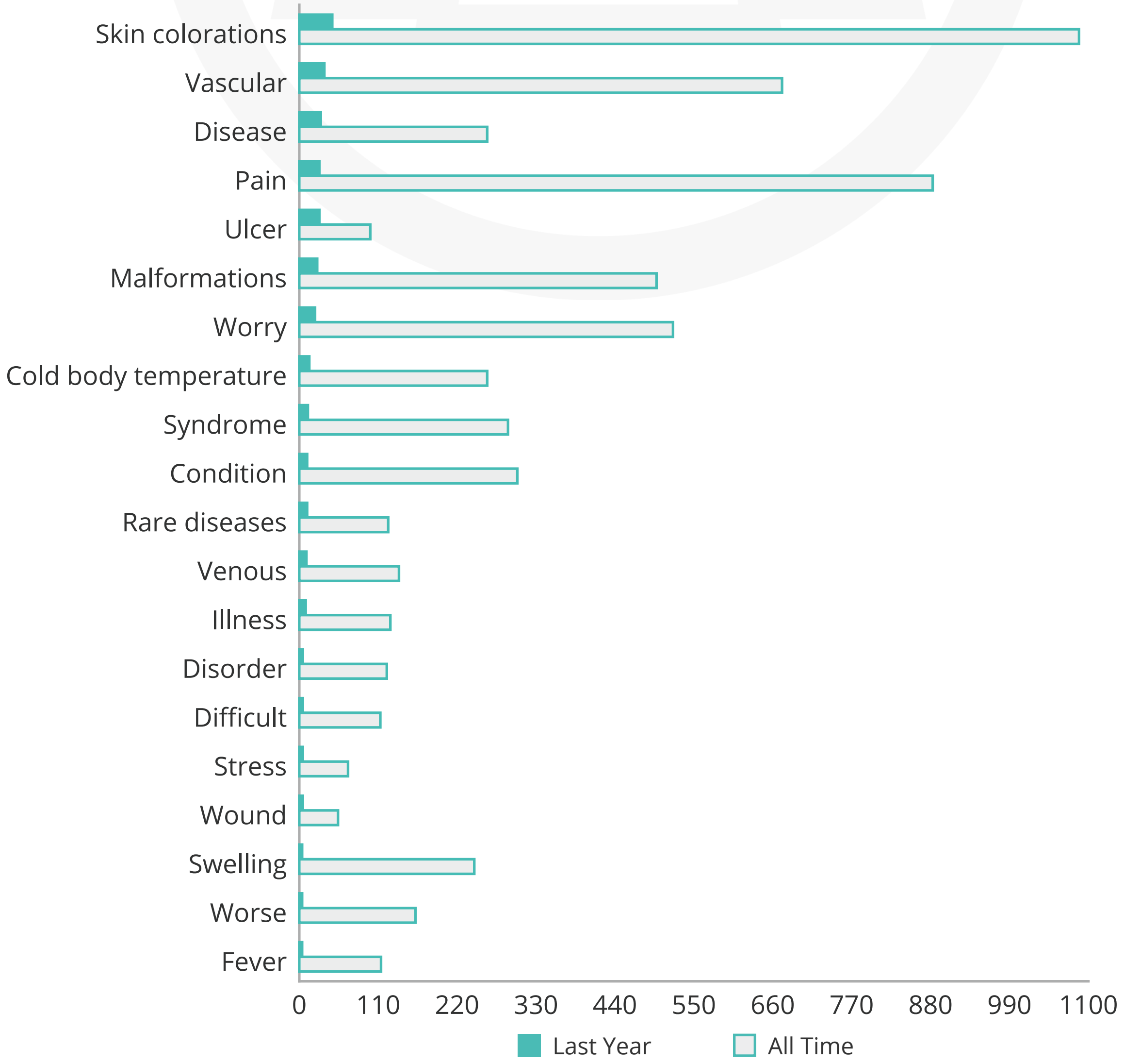
Terms to describe the psychological toll placed on patients and caregivers included *worry* (521/22) and *stress* (68/5).

The community supports people living with CMTC and similar vascular diseases. The disease names mentioned the most were as follows:

Disease Names Mentioned

• CMTC	3146
• CMTC-OVM	253
• M-CM	104
• Klippel-Trenaunay-Weber syndrome	82
• VM	55
• M-CMTC	24

DISEASE BURDEN: LAST YEAR VS. ALL TIME



This figure shows the number of mentions of concepts related to disease burden for a 12 months' worth of conversations and all of the conversations.

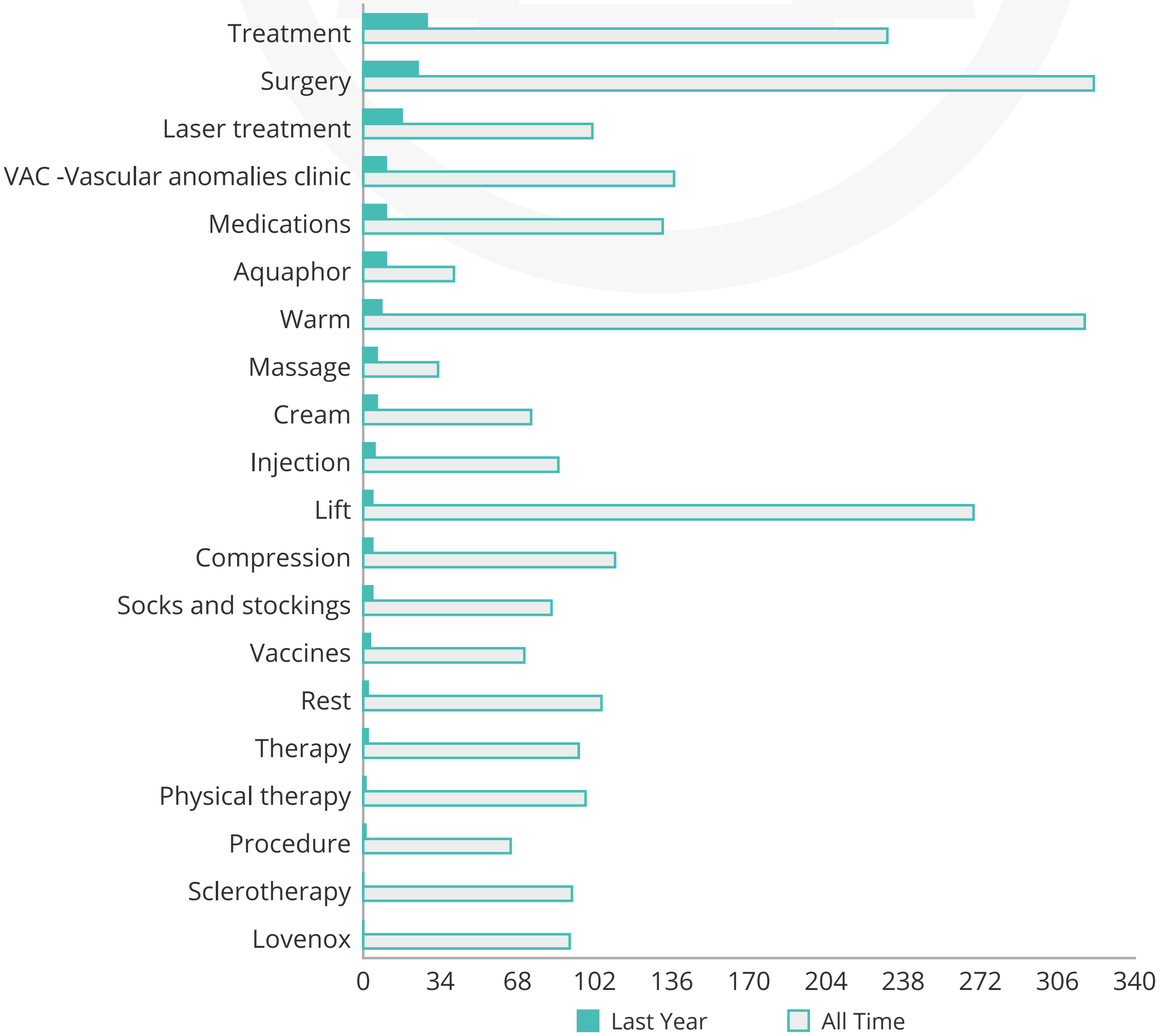
Disease Management

Treatments for the disease were discussed far less than the burdens of the disease. *Surgery* (322 all-time mentions/24 mentions in the last year) was often mentioned. The only drug mentioned with any frequency was *Lovenox* (91), which was not discussed at all in the last year.

Management techniques to offset the burdens of the disease were discussed, including *warm* (318/8), *lift* (269/4), *compression* (111/4), and *socks and stockings* (83/4). The analysis discovered that warm is associated with warming an affected limb to help with blood flow. Lift was usually mentioned as the medical device in shoes to help with leg length adjustments.

Aquaphor (40/10) and *cream* (74/6) were also mentioned.

DISEASE MANAGEMENT: LAST YEAR VS. ALL TIME



This figure shows the number of mentions of concepts related to disease management for 12 months' worth of conversations and all of the conversations.

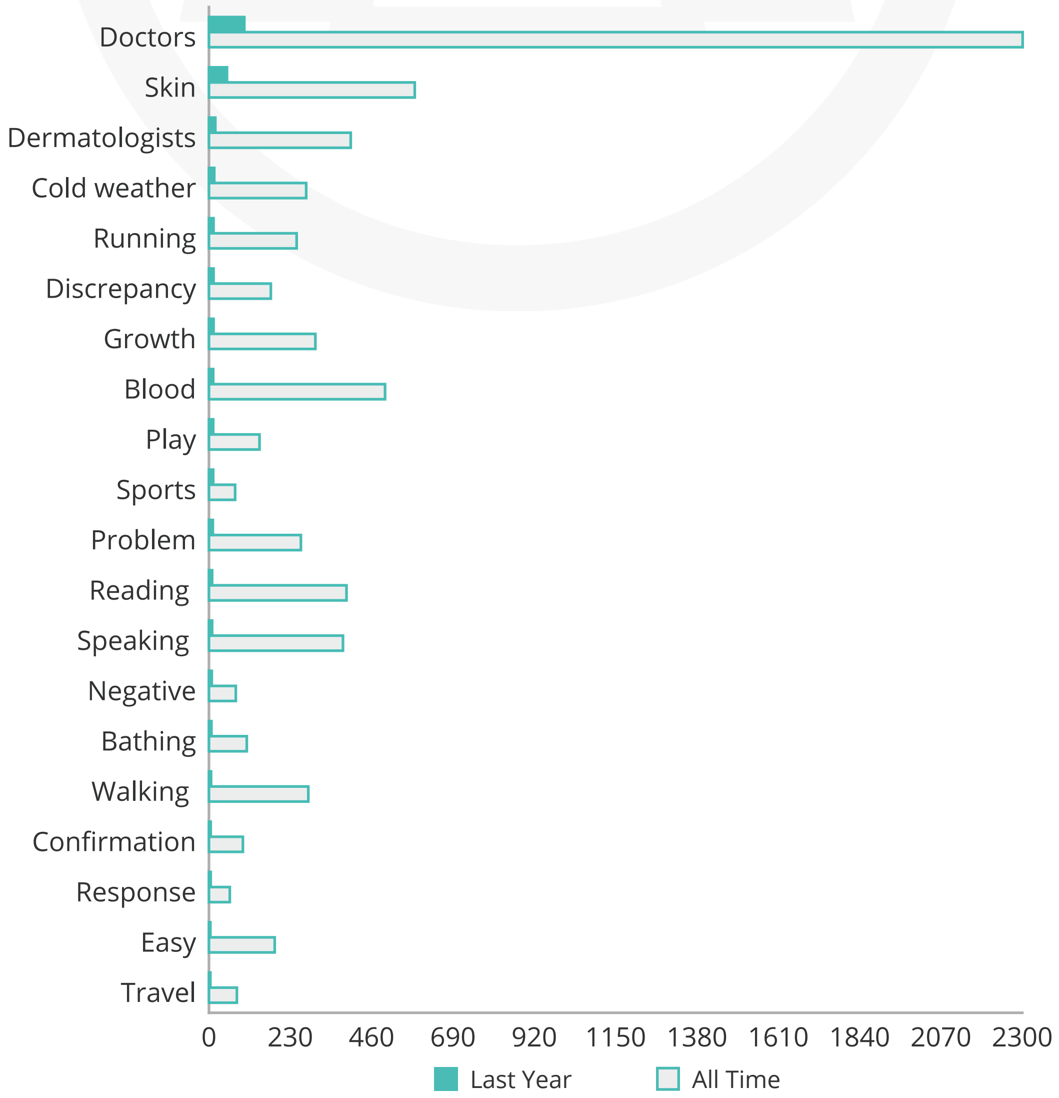
Significant Terms

Many terms discussed in the group are not easily identifiable as terms about the burden or the management of the disease. Some are common life terms such as “reading” or “speaking,” which often refer to reading about the disease or speaking to health professionals. Some such as “bathing” or “pressure” may be about burdens or management techniques. Further analysis would be required to understand the nuances within the conversations.

Growth (301 all-time mentions/13 mentions in the last year) came up frequently, but it was hard to tell what it was in reference to.

Doctors (2301/100) are frequently mentioned, with *dermatologists* (401/18) being the most talked about specialists.

SIGNIFICANT TERMS: LAST YEAR VS. ALL TIME



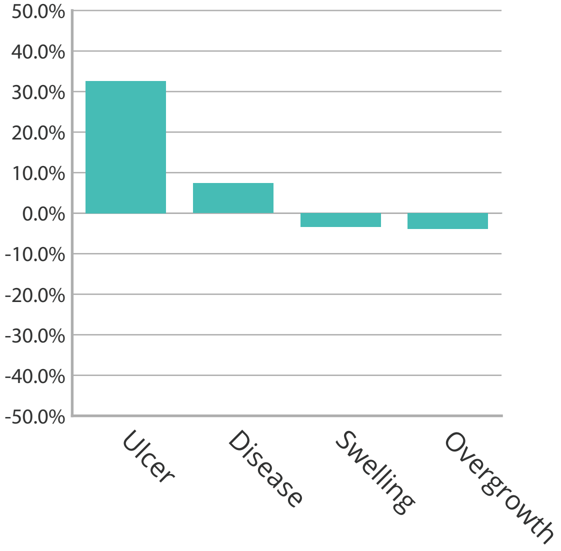
This figure shows the number of mentions of concepts that are significant but not clearly related to burden or management for 12 months' worth of conversations and all of the conversations.

Conversation Shifts Over Time

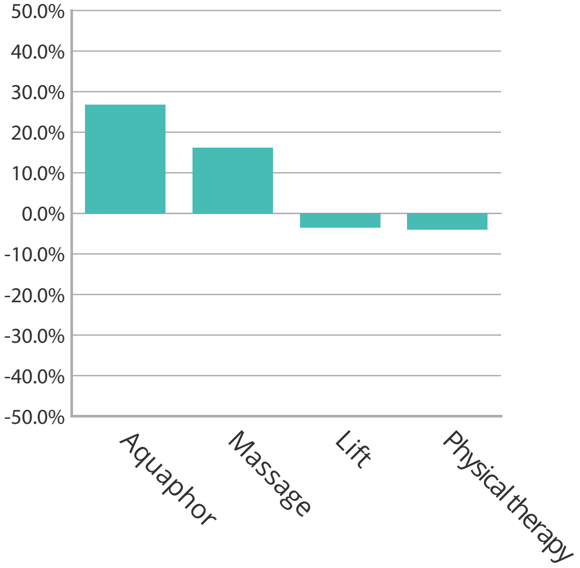
In our analysis of the data, we looked at the percent change in topics of conversation over the last 12 months compared to the entire time the participants have been active. There were fewer overall conversations in the last year than in the past. In the last year, 223 posts and 672 comments were made. Over the entire time the group has been in existence, an average of 250 posts and 1470 comments were made per year.

For Disease Burden, the concepts of *ulcer* (+32.6%) and *disease* (+7.4%) were mentioned more frequently in the last year. *Swelling* (-3.4%) and *overgrowth* (-3.9%) on the other hand, were mentioned much less frequently. For Disease Management, *Aquaphor* (+26.8%) and *massage* (+16.2%) were mentioned more frequently, whereas *lift* (-3.5%) and *physical therapy* (-4.0%) were mentioned less. In Significant Terms, *sports* (+13.5%) and *negative* (+6.2%) were mentioned more frequently, while *easy* (-2.8%) and *walking* (-2.9%) were mentioned less.

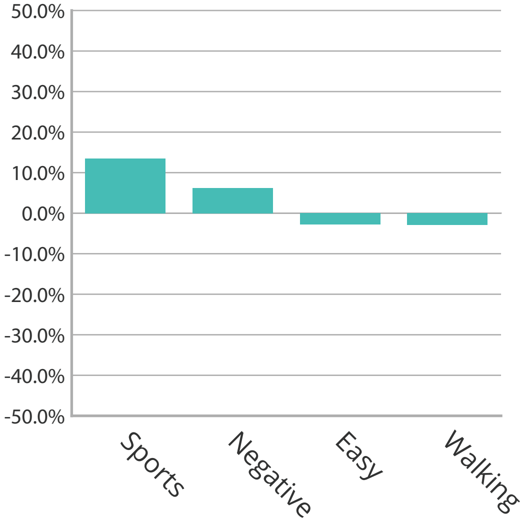
DISEASE BURDEN



DISEASE MANAGEMENT

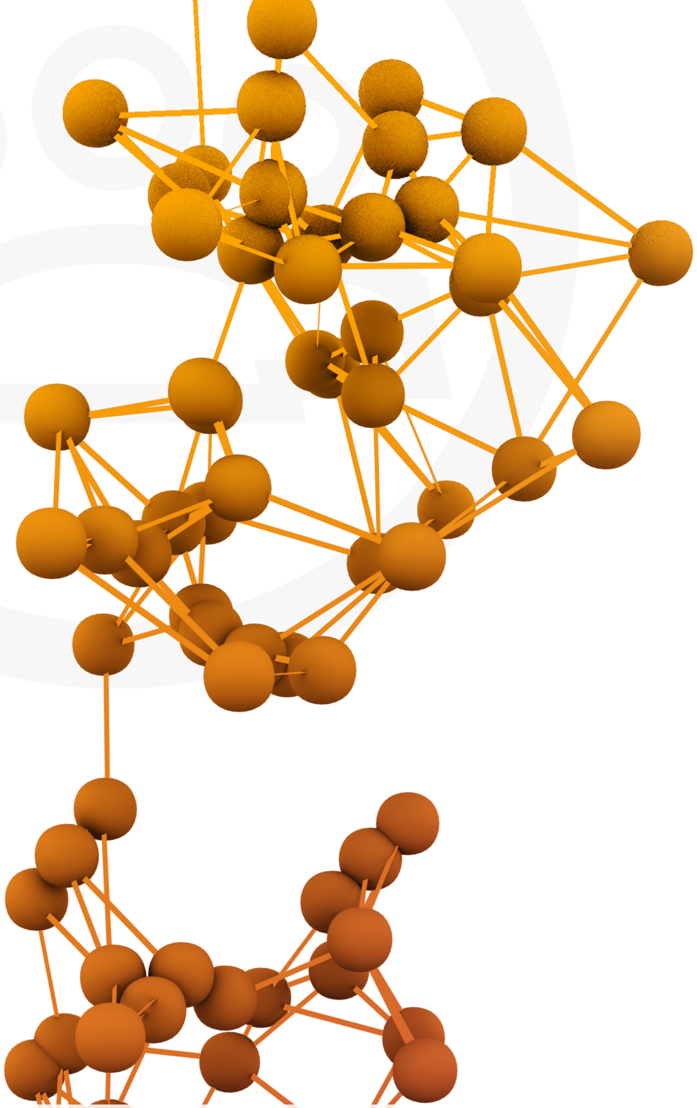


SIGNIFICANT TERMS



The above figures chart the percent change in mentions of concepts in the 3 areas of interest, burden, management, and significant terms.

Discovering Unmet Needs



As with many rare diseases, CMTC and other vascular malformations are experienced differently by each individual. Often the discussions in the group are questions about whether a particular symptom is related to the CMTC or not. Discussions also focus on how patients can best explain their illness to a doctor. This indicates a need for educational materials as well as more doctors who are qualified to provide expert medical care.

Members discuss differential growth in limbs and the resulting issues frequently. Patients and caregivers struggle to find shoes that will fit different sized feet. People exchange tactics for explaining size irregularities and the markings from CMTC. Dealing with bullies is also a topic of conversation.

Needles and vaccines were discussed in relation to how they can be administered in areas of the body affected by CMTC. Ulcers or wounds associated with vascular malformations became a more popular topic of conversation in the last year.

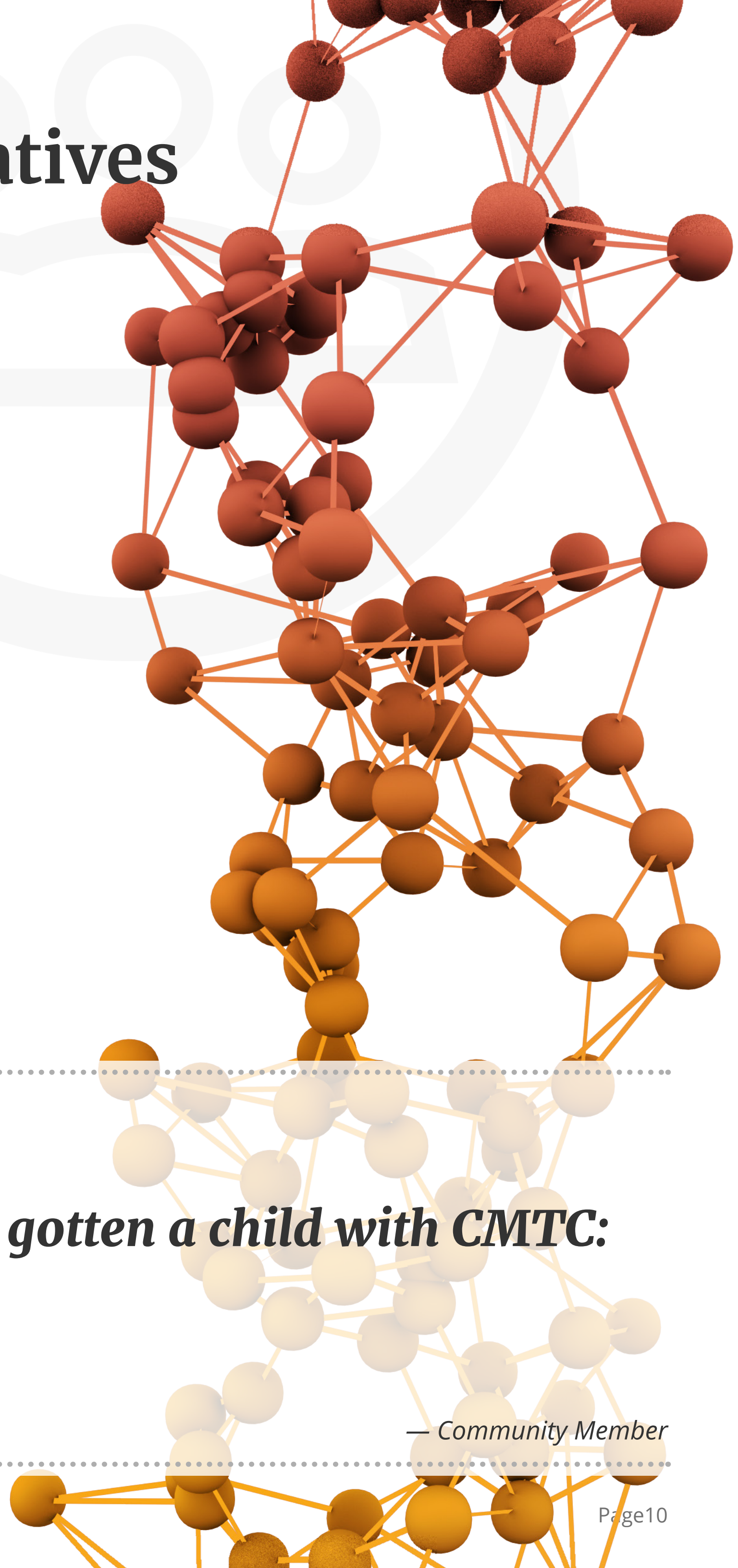
> ***“Speech therapy, physiotherapy and occupational therapy had a very beneficial effect on her. She got more confidence and dared more now that she could keep her balance better.”***
>
> *— Community Member*

Data Exploration and Health Initiatives

The data analyzed for this report revealed areas in need of further research. Because the community contributing to these discussions contains patients and caregivers dealing with CMTC and/or other vascular malformations, a Data Exploration could help define the symptoms of these different diseases and clarify the specific disorders under the umbrella term of "vascular malformations." Many of these variations are in need of further research.

Many people talk about issues they have with bearing weight on an affected leg. People discussed various ways to treat general pain and exploring these might help in the discovery of management techniques.

A Health Initiative could also help assess the best techniques, such as warmth and heating pads versus pain medications.



> ***"We also want to give this to parents who have just gotten a child with CMTC: try to enjoy them."***
>
> *— Community Member*

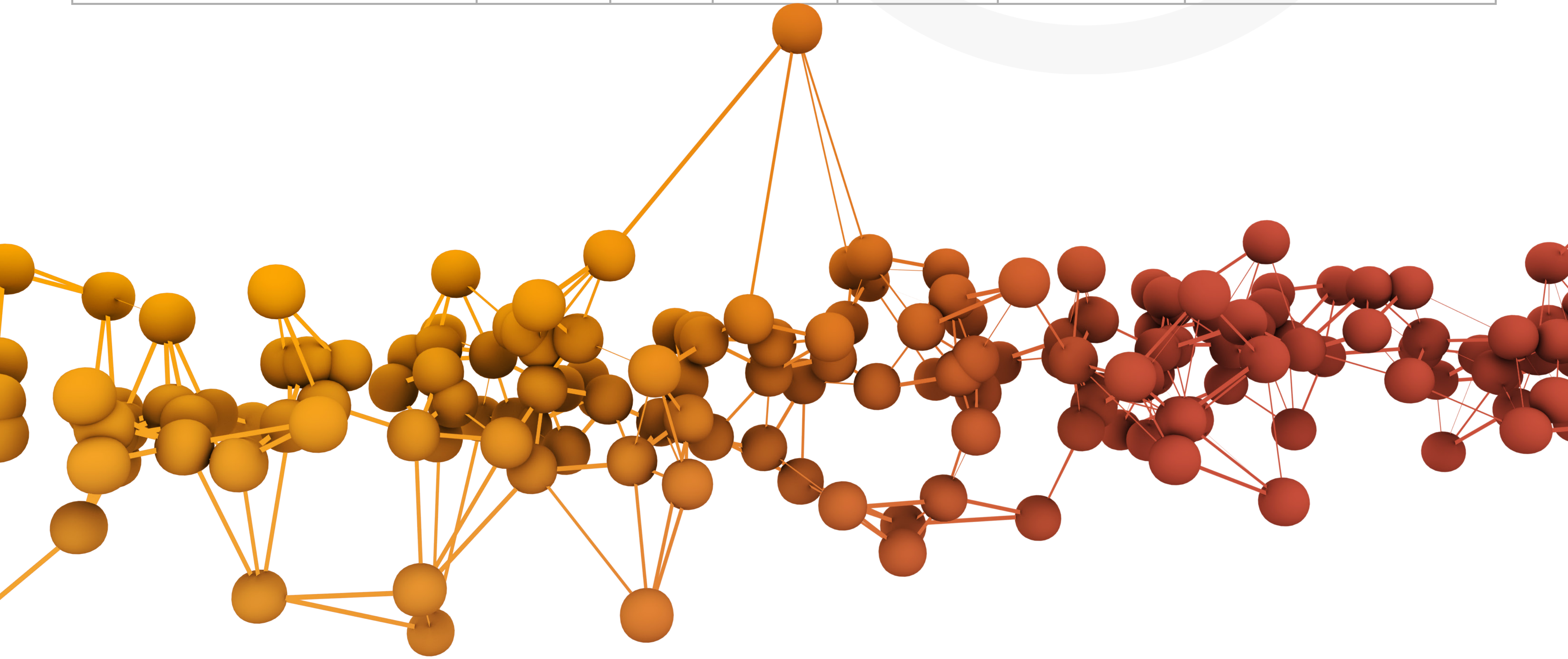
Appendix

Endnotes

1 https://rarediseases.info.nih.gov/diseases/6228/cutis-marmorata-telangiectatica-congenita

2 Itemized Table of Facebook Demographics

*Social Channels Analyzed	People	Posts	Comments	Gender	Age	Top 5 Countries
Facebook Group: *Global CMTC-OVM Organisation group*	780	2712	15424	Female: 83.0% Male: 17.0% Other: 0.0%	43.4% 35-44 Years	USA,UK,Netherlands,Canada, Australia



About TREND Community

Founders

TREND Community™ was founded by the parents of a child with Prader-Willi Syndrome who understand the needs of the rare and chronic disease community.

Our mission is to improve the quality of life for everyone living with rare and chronic disease.

Security

TREND secures all social data with state-of-the-art, private cloud servers. Our security practices comply with current HIPAA, FDA, and GDPR guidelines.

Disclaimer

The researchers who prepared this report are not doctors, are not providing medical advice, and are only reporting what was said in the online conversations.

IRB Exemption Status

Western Institutional Review Board determined that this study is exempt under 45 CFR § 46.104(d)(4), because the aim of the research is to collect de-identified information from social media posts to better understand disease burden, disease management strategies, quality of life, and the unmet needs of patient communities living with rare and chronic diseases. The research is not FDA-regulated nor classified, does not involve prisoners, and is consistent with the ethical principles of the Belmont Report.

Quotes

All quotes were provided by consenting community participants through one-on-one interviews or online focus groups conducted on the TREND Community™ platform. Names are not included to protect participant privacy.

Data Ownership

Ownership of public posts or conversations from other social media platforms shared with TREND for analysis are subject to the social media platform's privacy policy, terms of service and other applicable policies. Participants who share data and experiences on the TREND Community™ platform give their consent for TREND to analyze it. TREND Community owns the results of our analysis and all other data and output that we produce including our Community Voice Reports.

License

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