



# **PATIENT CENTRICITY AT CYTOKINETICS: IT COMES FROM THE HEART**

November 2021

“Our patients are a central thesis of our business.”

**ROBERT BLUM**  
CEO, CYTOKINETICS



On the cover: Employees at Cytokinetics honor American Heart Month during Wear Red Day

## OVERVIEW

**In this era of patient focused drug development and patient centricity, it is imperative for companies that make medicines to demonstrate true commitment to the people and communities they serve.** But there is a wide range of approaches among drug and biotechnology companies for realizing this imperative. For many companies, patient centricity is static, a “box to be checked” rather than a true commitment.

In contrast, for Cytokinetics, a late-stage biopharmaceutical company focused on improving the health span of people with devastating cardiovascular and neuromuscular diseases of impaired muscle function, patient centricity has been ingrained in the corporate culture since the company’s inception in 1997.

As part of the company’s Vision 2025, Cytokinetics moved to codify, systematize and increase awareness of its ongoing patient centricity efforts, publishing its value statement and action plan for delivering on the commitment to patient centricity:

**Patients are our NorthStar.** We build our science around patients and their families through authentic and ongoing engagement and are committed to transforming patients’ lives through our activities.

We embrace this at Cytokinetics by:

- ✓ **Striving to include the patient and caregiver voice.**
- ✓ **Encouraging a culture of compassion by affording opportunities for us to engage with patients.**
- ✓ **Understanding the burden of illness on patients and their families to inspire our discovery and development of potential medicines.**
- ✓ **Partnering with advocacy groups and patients to support and elevate patient communities.**
- ✓ **Realizing that patients are essential to our everyday decisions.**

This whitepaper presents a snapshot of how Cytokinetics is realizing its vision and delivering on this commitment, leading the field among pre-commercial companies, and demonstrating what can happen when “patient centrality is not a corporate strategy – but when it comes from the heart.” Of course, we recognize that this is a work in progress, with much more that can and will be done to elevate the company’s patient centric approach to new levels.

Lindsay Abromaitis-Smith, Artist and person living with ALS







John Crofut,  
member of Cytokinetics  
Heart Failure Patient  
and Caregiver  
Advisory Council

## CONTEXT: THE ERA OF PATIENT FOCUSED DRUG DEVELOPMENT & PATIENT CENTRICITY

**Since the beginning of medicine, the goal of developing therapies has been to benefit the patient.** Yet the complexity of this work has spawned a system where those developing treatments are often disconnected from the patients they serve. As a result, until relatively recently, direct industry interaction with patients has historically been infrequent, episodic, and generally limited to late-stage clinical trial recruitment and post-approval marketing.<sup>1</sup>

This dynamic is changing dramatically, as patients have moved “from passengers to co-pilots” in the medicine development journey. Many of today’s patients have embraced the mantra of “nothing about us without us,” as they seek a proactive, engaged, and central role in their health care,<sup>2</sup> bringing their personal perspectives into every aspect of drug development.

There is emerging consensus among patient advocates, industry leaders and regulatory officials about how patient engagement early and often throughout the therapy development continuum can provide significant value to all stakeholders. Specifically, the increasing focus on implementing Patient Focused Drug Development (PFDD) practices reflects the trend towards strategic integration of patient perspectives.

Patient insights– such as how they might make trade-offs between potential benefits and risks, and how they define meaningful outcomes for their specific conditions– are incorporated in the development process to help improve the quality and efficiency of clinical trials, and ultimately lead to more effective and meaningful therapies for patients.<sup>3 4 5</sup>

The term “patient centricity” can mean different things to different stakeholders. Most published definitions include some variation on the theme of “putting the patient first in an open and sustained engagement to respectfully and compassionately achieve the best experience and outcome for that person and their family.”<sup>6</sup> But more important than the words used to describe what it means are the actions undertaken to make it real.

**Larry Williams,**  
member of Cytokinetics  
Heart Failure Patient  
and Caregiver  
Advisory Council





The “Wall of Heroes” unveiling at company headquarters celebrates patients and families

## EMBODYING PATIENT CENTRICITY AT CYTOKINETICS

**At Cytokinetics, the goal is to embed patient centricity into every aspect of the corporate culture.** Fred Fisher, the CEO of the ALS Association Golden West Chapter and long-time advocacy partner, shared that Cytokinetics demonstrates “consistently genuine, empathic concern for the population the company is trying to help.”

In its public-facing materials, Cytokinetics describes its focus on developing a community of colleagues who are impassioned by their purpose to improve the lives of patients. Employees can contribute to something bigger than any individual, as every person contributes something unique to the shared corporate goal of improving the lives of people with diseases characterized by muscle dysfunction, weakness, or loss.

The company’s values statements outline what is important and what is expected. The foundation for everything the company does is patient centricity. Recognizing it has set high expectations, especially given its current pre-commercial status, Cytokinetics challenges its team to lead with science and deliver for patients. Through this corporate culture, “everyone is motivated to do something that will change someone’s life,” says Kenna Reehil, Senior Director of Global Marketing.

“Commitment to patients is a powerful force within the bloodstream of the company.”

**ROBERT BLUM**  
CEO, CYTOKINETICS



The goal is to change the lens of how people think about their jobs and help them understand that every member of the company has an impact on the patient. Through a series of internal processes and activities designed to reinforce the clarity and transparency of the company's goals and values, Cytokinetics' employees across all levels of the company, are incentivized and supported to embody and personify patient centricity, always and all ways:

- ✓ **Employee goal setting includes demonstration of patient centricity.**
- ✓ **Everyone is expected to engage in patient advocacy.**
- ✓ **Staff are provided paid time off for community engagement.**
- ✓ **All staff are encouraged to make personal connections to ensure that they know what the disease looks like “face-to-face.”**
- ✓ **Patients and caregivers are regularly invited in to meet with employees.**
- ✓ **Corporate offices include visual reminders of the importance of the lived experience of patients.**

Jessica Hall,  
member of Cytokinetics  
HCM Patient and  
Caregiver  
Advisory Council





Cytokinetics scientists, Makoto and Luke, share the company's commitment to serving the patient community

## PATIENT CENTRICITY IN ACTION AT CYTOKINETICS: SNAPSHOT OF IMPACT

“Thank you for taking the time to really listen!”

**LINDSAY DAVIS**  
COUNCIL MEMBER,  
CYTOKINETICS HCM PATIENT AND  
CAREGIVER ADVISORY COUNCIL



### Patient Centricity in Research: Understanding and Meeting Unmet Need

One of the most patient-centric aspects of drug development is ensuring that research programs are planned and executed based on an understanding of the true needs of the patient community.

Cytokinetics commits itself to a disease state rather than a single drug or trial outcome. This approach can be seen in the company's cardiovascular and neuromuscular programs, where Cytokinetics has repeatedly made decisions about research based on the needs of the patients.

For example, in heart failure (HF), when its partner in development opted to end the collaboration for business reasons, Cytokinetics recognized the importance of delivering a different kind of therapy for the treatment of HF and chose to continue its program, despite the additional costs and risk of doing so. In hypertrophic cardiomyopathy (HCM), while the company is developing a therapy for today's patients through a series of clinical trials, it is also investing in research to understand the inherited



risk component of the condition (based on a genetic defect) to address the impact HCM has on families as patients are concerned about what to do if their child carries a mutation.

This disease focus is also clearly on display in Cytokinetics' amyotrophic lateral sclerosis (ALS) program, where a heartbreaking failure in a later stage trial catalyzed the company to double down. Where many companies would have chosen to abandon the program, Cytokinetics made the moral and ethical decision to continue to make the investigational therapy available on a compassionate use basis to patients who had participated in the clinical trial through a managed access program. Additionally, following through on its commitment to the disease and the patient community, the company went back to the research drawing board to understand why the trial failed and apply those lessons to the next generation drug candidate already in development. This persistence has materialized into the start of a new phase 3 study with *reldesemtiv*. The study, COURAGE-ALS, received the highest possible patient centricity rating based on criteria developed by an ALS patient advocacy group: <https://iamals.org/patient-centric-trial-design-pactd-rating-criteria/>. As described by an advocacy leader in the ALS community, while other companies would have “ghosted us,” Cytokinetics demonstrated perseverance to deliver.



I recognize that the drug might not succeed. In that case, I expect that they will apply what they have learned to move forward toward success. That's their mindset."

**SCOTT POPJES**  
COUNCIL MEMBER, CYTOKINETICS HCM PATIENT AND  
CAREGIVER ADVISORY COUNCIL

## **Patient Centricity in Clinical Development: Making Trials Easier for Patients by Incorporating Patient Voice**

Patient focused drug development is predicated on efforts to generate and incorporate patient insights into clinical trial design and operations. Cytokinetics is implementing a holistic approach to these activities. In HCM, HF, and ALS, the company has convened Patient and Caregiver Advisory Councils (PACs) for ongoing engagement of people impacted by these diseases rather than the more traditional “one-off” patient advisory boards. By continuing to engage the councils over time, the company develops deeper relationships and understanding of patients’ needs. This informs the planning and conduct of clinical trials, as well as other areas of business such as preclinical and commercial planning. Related to trials, PACs provide input on ways to make clinical trial protocols easier on patients, including such aspects as remote visits, fewer tests, reimbursements for travel, stipends for childcare or eldercare.

**Nefertari Nelson Williams,**  
member of Cytokinetics  
Heart Failure Patient and  
Caregiver Advisory Council





“The only way to get good information is to be quiet and listen. We ask patients and their family members about ‘what in your life are you missing that you would like to have back?’”

**MARY POMERANTZ,**  
**DIRECTOR OF ADVOCACY, CYTOKINETICS**

Determining what to measure in a clinical trial requires a deep understanding of what truly matters to patients and what they value most. This goes beyond evaluating the traditional clinical measures that a physician will record. While overall survival is considered the gold standard metric by regulators for certain disease states, there can be other important indicators of a therapy’s impact on patients lives, including how they feel and function. Through validated Patient Reported Outcome Measures (PROMS), clinical studies can evaluate how patients feel and function, by asking them to report directly on a series of factors that have been identified to be both important to them and relevant to the impact of the therapy under study. For example, cardiovascular patients may value fewer trips to the hospital, less fatigue, or the ability to exercise longer, elements that can be measured using various PRO assessment tools.<sup>7</sup>

Moving beyond convening advisory councils and beginning with its 2021 HCM Phase 3 study and its Phase 3 ALS study, Cytokinetics has invited patients to serve as members of clinical trial steering committees, further embedding the patient voice into each critical phase of the development program. The company is also committed providing appropriate trial updates and information about results to the advocacy community. This type of ongoing communication and feedback that treats patients as partners is not generally provided by most companies, despite being a key indicator of patient centricity.



“It is incredible what patients are teaching us. We owe it to them to be their partners”

**PATRIZIA ALLEGRA**  
SENIOR DIRECTOR,  
MARKETING - RELEDESEMTIV,  
CYTOKINETICS



In all cases, Cytokinetics has worked to make it as seamless as possible for patients and advocates to partner with the company and appropriately honoring their role. Robin Sparks, Executive Assistant at Cytokinetics recognizes that “this can go well, or this can be ‘cringeworthy’” by diminishing the value of a patient advisor, and she helped the company refine its contracting process. The updated process uses appropriate language that respects their role and places patients on the same footing of importance as scientific advisors and healthcare providers. Jacqueline Lee, Senior Director, Clinical Operations at Cytokinetics also noted the importance of recognizing patients as equal partners.

### **Patient Centricity in Commercial Activities: Patients are the Most Important Stakeholder**

As a pre-commercial company, Cytokinetics is demonstrating the importance of building the foundation for a patient-centric commercial approach, well before the point of having a product in the marketplace. The company’s mindset is that there are ways to do what is best for the patient AND differentiate the company in the marketplace.

This effort includes recognizing that the most important stakeholder for the company is not the physician, but rather is the patients and their caregivers (those directly impacted by a condition or disease.) Cytokinetics is building its approach to brand development on efforts to know these most important customers and truly understand the full range of their needs. In addition to assessing the safety and efficacy of a drug, the company is also focused on best approaches for formulation, administration, dose, and schedule to best meet patients’ needs.

For example, in its HCM program, Cytokinetics is working with patients and caregivers to understand preferences that will inform pill shape, color and packaging, while identifying common challenges with medication management.

Thinking ahead to the time when Cytokinetics has its first commercial product, the company is laying the foundation for a patient-centric approach to access and affordability. By establishing a group of HF ambassadors to share their journey with payers and pharmacy benefit

managers, the company is using its business platform to raise awareness of heart failure among healthcare stakeholders. This can be especially impactful for administrators who often are removed from the direct patient experience. To the extent possible, the company is focused on delivering a “total patient solution” that creates an “umbrella of care” for the patient.

## **Patient Centricity in Community Support: Going Beyond the Business Imperative**

Leveraging patient advocacy to support the patient communities that are the focus of a company’s therapy development pipeline is a critical aspect of patient centricity. This type of engagement ensures that company officials and programs are well-versed in the needs and perspectives of patients and families impacted by a specific disease or condition.

Employees, family and friends participate in Team Cytokinetics at the Napa Valley Ride to Defeat ALS put on by the Golden West Chapter of the ALS Association







Advocates Chris and Liz Mavraedis with the late Ryan Farnsworth at a company awareness and fundraising event for ALS.

Patient advocacy at Cytokinetics is a robust, company-wide program of engagement that crosses functions to support and engage the communities the company serves. The company views its partnerships with patient advocacy organizations and other non-profit entities as relationship-based rather than transactional. Engagement means more than providing financial support, as key departments are represented in Cytokinetics' decision-making and outreach with the advocacy community.



Cytokinetics company-wide event to honor all families in the fight against ALS... is what we mean when we say 'patient centric.' Thank you for encouraging me to share my story."

**GWEN PETERSEN**  
COUNCIL MEMBER, CYTOKINETICS ALS PATIENT AND  
CAREGIVER ADVISORY COUNCIL





Cytokinetics honors the ALS community at a Giants game in San Francisco near company headquarters

The company is transparent about its holistic approach, defining its patient advocacy objectives to include:

- ✓ **Elevating Patient and Caregiver Voices**
- ✓ **Driving Public Policy Advancements**
- ✓ **Championing Better Care, Access, and Patient Services**
- ✓ **Raising Awareness and Increasing Understanding of the Burden of Disease**
- ✓ **Supporting Fundraising Efforts and Community Education**

These activities and areas of focus are applied broadly, within communities that represent the disease areas of relevance to the company's broad portfolio aimed at cardiovascular and neuro-muscular conditions.

In the cardiovascular space, the company “punches above its weight” in providing funding to a range of advocacy and non-profit organizations, choosing to provide longer term support to help build the community wherever possible by supporting patient and caregiver education and awareness, as well as support for capacity building initiatives (such as fundraising, marketing and communications). For example, in partnership with a leading cardiovascular non-profit, Cytokinetics is co-developing



a program to enable a smooth transition from hospital to home for patients managing heart failure. Finding the right physician specializing in heart failure management can be a challenge for patients. That's why the company is supporting a Physician Finder tool on an advocacy organization's website to facilitate the journey toward optimal care.

Within the neuromuscular arena, Cytokinetics sees its role as focusing on the entire ecosystem around the patient. For example, within ALS the company not only provides critical funding support to patient advocacy organizations to provide care services for people living with ALS and their caregivers, but also has prioritized efforts to provide public policy support on issues important to the ALS community. This approach to maintaining community ties through advocacy is also on display within the SMA space, as the company continues to provide funding support even though its SMA development program is currently paused in order to prioritize more advanced pipeline programs.

**Employees participate in virtual candle lighting ceremony during SMA Awareness month in 2020**







Cytokinetics employees join the global ALS and motor neurone disease community at a fundraising and awareness event in Perth Australia

## A WORK IN PROGRESS: FUTURE DIRECTIONS FOR CYTOKINETICS

“We will keep asking ‘what can we be doing better?’”

**ROBERT BLUM**  
CEO, CYTOKINETICS

**Cytokinetics is focused on expanding and deepening its patient centricity efforts, as its leaders recognize that more must be done, and the company cannot afford to become complacent about its efforts to date.** The company is clear-eyed in recognizing that its patient centricity focus “rubber will meet the road” as it nears commercialization of its first product. Decisions about pricing, patient support and additional development programs will be made and must be evaluated based on how they impact patients and families.

Working with its employees and key stakeholders – including its board of directors and advisors – Cytokinetics is continually challenging itself to be truly patient centric. Based on an abiding sense of personal and professional responsibility to others, the philosophy of “doing well by doing good” remains a positive example of what a for-profit company can do to meet the needs of its constituents.

These efforts are a work in progress. This story is to be continued...



# NOTES

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