September 9-10, 2024

RECAP REPORT

Top of Mind Summit: Life Sciences

Center for **Connected** Medicine

UPMC Enterprises

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Top of Mind Summit: Life Sciences

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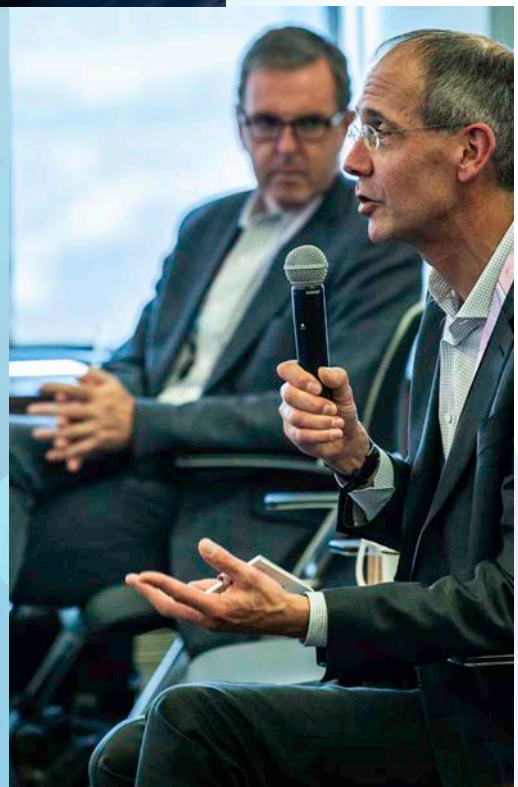


The second annual Top of Mind Summit: Life Sciences was held on September 9-10, 2024, at the Center for Connected Medicine at UPMC (CCM).

The conference, organized by UPMC Enterprises and the CCM, brought together approximately 100 thought leaders in health care, biotechnology, government policy, pharma, and the life sciences to discuss and debate existing and emerging challenges and opportunities in the life sciences and health care ecosystems.

This year's Summit centered on the convergence of technological innovation, patient care, and how successful collaborations in the life sciences can catalyze the rapid evolution of new models in health care that enable life changing medicine. The Summit focused on harnessing real-world data and artificial intelligence (AI) to improve the therapeutic development process, transform how clinical trials are designed and conducted, and how to more efficiently and equitably deliver health care services and novel, innovative therapeutics. Throughout the event, the panel discussions highlighted the growing importance of partnerships between health care systems, academia, and industry in driving continued innovation while advancing health care access and equity.

New for this year's conference was a thematic symposium dedicated to women's health. The goal of the women's health-focused symposium was to convene a network of committed partners across the spectrum of women's health research, access, and investment to establish a knowledge base, discuss critical issues, and explore tangible action items to make real progress in improving health care for women. A detailed summary of the symposium, including key takeaways and next steps, will be published separately.

Summit Themes and Highlights

Across the various panel discussions, several shared themes and cross-sector challenges emerged that apply in some way to the entire spectrum of how health care and health care innovations are designed, tested, and put into clinical practice. As health care continues to evolve, the discussions from the Summit serve as a guide for how stakeholders across the industry can work together to ensure that the benefits of innovation are accessible, affordable, and equitable.

Stakeholders Connected by Patient-Centric Solutions

The symposium highlighted the importance of strategic collaboration between health care providers and payers, academic research institutions, and innovative life sciences industry players. These partnerships are essential for tackling the multiple complex challenges faced by the health care industry, particularly in balancing therapeutic innovation with access and the equitable use of scarce resources. Aligning incentives across these stakeholders is not always easy and requires a shared, patient-centric vision and long-term commitment to succeed. Breaking down information silos and integrating real-world data into decision-making will serve as a bridge between key stakeholders. This integrated approach will create a path for developing groundbreaking insights, and spur collaborative partnerships and strategies to develop innovative treatments and advance patient care discoveries.

The Inevitable Role of AI in Health Care

AI emerged as a central theme throughout the Summit, with many speakers emphasizing its transformative potential to harness the vast amounts of data generated daily by health care institutions and allied partners. AI's ability to process, analyze, and deliver insights from real-world data offers the promise of revolutionizing research, therapeutic discovery, and patient care. By accelerating hypothesis generation and enhancing decision-making, AI can enable more precise, data-driven health care, streamlining processes that traditionally relied on time-consuming manual analysis.

However, fully realizing AI's potential in health care requires sorting out complex challenges, such as data standardization and the creation of environments that facilitate the safe and ethical sharing of currently siloed data. Summit speakers highlighted the importance of collaborative partnerships between stakeholders — health care systems, industry, researchers, and regulators — to spark innovation while ensuring ethical oversight. As AI continues to evolve in its own right and how it may be used in health care settings, the technology inevitably will serve as a foundational tool for intelligent health care, unlocking new insights and advancing the development of innovative treatments and therapeutic modalities hidden within the collective data we have generated.

Accessibility and Affordability in Health Care Delivery

The ongoing challenge of making cutting-edge therapies accessible and affordable without stifling innovation or compromising care is top of mind for many stakeholders. New financial models, such as value-based care, are potential solutions to balance the competing priorities of innovation and cost control, but there needs to be consensus to make this dynamic work. These conversations and themes extended to addressing entrenched disparities in health care access, with UPMC and its unique approach and integrated system configuration seen as a potential model for establishing pilots and creating systems that can lead the way in solving these issues through greater alignment of incentives.

Improving Clinical Trials for Greater Impact

Improving the clinical trial process — particularly in patient engagement and diversity — emerged as a central theme across multiple panel discussions. An evolving model suggests that real-world data and the use of AI can help to make clinical trials more efficient by identifying potential participants more quickly and optimizing trial designs. Achieving this vision requires rethinking the traditional approach and calls for closer collaboration between health care providers, industry sponsors, and regulatory bodies.

Large health care organizations, such as UPMC, are well-positioned to provide tremendous value to trial sponsors via closer, more direct partnerships. By integrating trials into the day-to-day care environment, provider systems can help reduce barriers for patient participation and improve diversity within studies, while making trial outcomes more reflective of real-world health dynamics and needs. Health care systems can leverage their extensive EHR data and AI tools to identify eligible patients more precisely, leading to faster recruitment and meeting target accrual goals through a more streamlined trial process. Investments by health care systems and trial sponsors in this model not only facilitate direct patient access to trials, but also strengthens trial governance by aligning sponsor objectives with clinical realities. This collaborative approach supports trial designs that prioritize patient-centered methods, such as remote monitoring and flexible visit schedules. The resulting improvements in trial design and outcomes stand to benefit all stakeholders — patients, providers, sponsors, and health systems — while laying the foundation for a more efficient and responsive clinical trial framework that delivers meaningful insights reflective of a diverse population.

Opening Keynote:
Enabling Life-Changing Medicine

Jeanne Cunicelli
Executive Vice President, UPMC; President, UPMC Enterprises

Jeffrey Solomon
President, TD Cowen;
Member of the UPMC Board of Directors

"Change is constant — this is true in life and most certainly in health care. UPMC has always embraced change, innovation, and risk-taking, qualities that have translated into a successful track record of advances in health care delivery."

The opening talk at the 2024 Top of Mind Summit: Life Sciences between Ms. Cunicelli and Mr. Solomon highlighted UPMC's ongoing commitment to innovation, investment, and strategic partnerships to advance health care. The discussion focused on UPMC Enterprises as a driving force behind the health system's ability to dispose of scientific and clinical risk to translate cutting-edge research into practical, impactful health care products and therapeutic solutions that can improve patient care.

They emphasized the need for health care systems to continuously adapt to change while maintaining a long-term vision that prioritizes accessibility and affordability alongside innovation — an approach at which UPMC excels — by bringing together top talent and fostering collaborations that extend beyond traditional health care boundaries. This forward-thinking philosophy has allowed UPMC to take intelligent risks and focus on scalable solutions that meet the needs of a diverse patient population. Clinical expertise, academic research, intelligent risk-taking, and industry partnerships are all required to solve complex health care challenges, particularly as UPMC continues to push at the frontiers of translational science.

The discussion also touched on the necessary balance between pioneering treatments and ensuring that they remain accessible and affordable to a broader patient population. Unaffordable treatments or care pathways that limit access are inefficient and waste the significant capital and human expertise used to create them, even if they are efficacious in a small subset of patients lucky enough to have access. Ms. Cunicelli and Mr. Solomon emphasized that achieving this balance is essential for delivering life-changing medicine and lasting impact on long-term health outcomes.

UPMC's strategy of leveraging innovation, collaboration, and a community-centered approach serves as a model for how health care systems can push the boundaries of medicine while staying focused on improving patients' lives in a sustainable and equitable way.

"Investment in community institutions translates into meaningful advancements in patient care. The question is how we can structure these investments to make substantial, lasting changes in medicine."

The Long View: Creative Partnering to Solve Big Problems

Steven Altschuler
Managing Director-Healthcare Venture,
Ziff Capital Partners

Matthias Kleinz, DVM, PhD
Executive Vice President of Translational Sciences,
UPMC Enterprises

David Moore
Executive Vice President, Corporate Development,
Novo Nordisk

Jonathan Peacock
Chair of the Board,
Bluesphere Bio, UCB, and Avantor

The day's first panel discussion emphasized the necessity of long-term partnerships to address the most complex problems in health care. This session explored how long-term partnerships, strategic risk-taking, and perseverance are essential ingredients for bringing innovative health care solutions to market. The panelists, all seasoned health care and biotech leaders, discussed the importance of combining innovative science with a

> **"Investing is about finding the people who can move things forward, those with the vision and entrepreneurial drive to bring new frontiers to life."**

deep understanding of patient needs and market access. Creating successful biotech ventures and other life-changing solutions in health care often requires a long-term vision and deliberate approach of patiently deploying capital that supports projects through long development cycles and setbacks before demonstrating clinical value and providing the desired returns. In essence, "slow and steady wins the race."

One theme that emerged during the talk was the potential for unique collaboration opportunities between industry leaders, clinical institutions, and academic centers. These partnerships have the ability to connect academic rigor with large-scale industry resources, enabling companies to invest in basic scientific research, which is the foundation for innovation. An example of this type of approach is Novo Nordisk's academic partnerships to study and create solutions for cardiometabolic disease. This type of collaboration can lead to a greater understanding of the complexity of human molecular and cellular biology, which can then be translated into concrete drug discovery and development efforts. By fostering academic-industry connections, the health care sector can bridge its work in basic science with real-world therapeutic applications.

The discussion also underscored UPMC's distinctive approach to innovation. The industry experts convened for this discussion highlighted their decisions to partner with UPMC Enterprises because of how the organization combines clinical and scientific insights with the discipline of an investment fund to create innovation and change in health care. The UPMC model does not just fund projects — it curates initiatives that align with its mission to advance patient-centered health care. This synergy between UPMC's capabilities as a medical center and payer, and its focus on thoughtful investment through UPMC Enterprises, allows partners to confidently support breakthrough solutions with the infrastructure to scale those advances effectively.

Another point of discussion stressed the importance of investing in human capital, noting that leadership plays a central role in navigating the uncertainties inherent in the biotech innovation process. As is often the case in the realm of biotech innovation, novel drug discovery, and clinical care advancements, there's a razor thin line of demarcation between what succeeds and what ends up in the bin of failures. In no small part, what can push an idea or innovation across that line to the side of success is the make-up of the team itself — its leaders, champions, and experts, and its partners.

Open engagement with seasoned advisors and cross-disciplinary collaboration leads to resilient leadership and adaptability — qualities that are needed in large measures to navigate the uncertainties of the health care innovation process. Investing in skilled, adaptable teams provides the backbone for these ambitious projects, helping translate promising concepts into impactful solutions for patients.

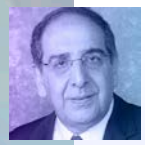
Maintaining a long-term strategic vision while embracing "smart risks" can lead to meaningful solutions to health care challenges.

Getting Close & Personal I: Strategies for Efficient Trials and Enhanced Patient Engagement

Nicole Ansani
Senior Vice President, New Development Initiatives,
UPMC Enterprises

Beth Brooks
Head, Patient Insights and Behavioral Sciences,
Sanofi

Simon Dagenais
Senior Director, Real World Evidence,
Pfizer



José-Alain Sahel, MD
Chair and Distinguished Professor,
Department of Ophthalmology,
University of Pittsburgh School of Medicine

Anantha Shekhar, MD
Senior Vice Chancellor for the Health Sciences
and Dean of the School of Medicine,
University of Pittsburgh

This panel focused on the challenges of conducting clinical trials and the need to improve many aspects of this fundamental pillar in developing safe and effective treatments. The current model of how clinical trials are designed and conducted continues to be plagued by inefficiencies in design, cost, speed, patient engagement and retention, and data quality. The fact that 80% of clinical trials fail to meet their recruitment targets, leading to costly delays and suboptimal data, is the one statistic that ought to convince all stakeholders that systemic changes must be brought to bear in how clinical trials are designed and conducted.

“The key to improving clinical trials is integrating them into regular clinical practice, making it easier for patients to participate and for researchers to conduct meaningful studies.”

The discussion highlighted the importance of direct patient engagement facilitated by large health care systems, such as UPMC, which can communicate with patients through established patient portals and other existing communications infrastructure rather than relying solely on dedicated trial tools or apps. This approach leverages platforms or technology that patients already trust and use regularly when discussing or planning their health care needs with their providers. In addition, the use of financial incentives, logistical support, and clearer communication about the value of clinical trials were suggested as tactics that could potentially remove or mitigate known or probable barriers to increased participation. Increasing awareness is also an essential ingredient; without knowing that clinical trials exist, or what the potential benefits are, patients are unlikely to enroll or stay engaged throughout the process. The same holds true for providers in the system. If a patient’s health care practitioners are not equally informed about existing trials or their potential clinical efficacy, a trusted voice for encouraging participation and engagement is equally silenced.

Increasing diversity in clinical trial recruitment is another area in which substantial change needs to occur, particularly in ensuring that trials reflect broader populations, including women and minority groups, to make the data and findings more universally applicable or targeted.

AI and real-world data are emerging as powerful tools to streamline trials. By optimizing patient identification and using virtual models, AI can reduce costs and make trials more accessible.

“Delays in clinical trials are costly, and we’re facing serious challenges with recruitment. We need to rethink how industry and medicine collaborate to overcome these barriers.”

The panel concluded that the best way to transform clinical trials is through closer collaboration between health care systems and industry sponsors. A more patient-centered trial model, supported by partnerships that align clinical realities with sponsor objectives, has every possibility to improve trial outcomes, accelerate the development of new therapies, and ensure that trials are conducted sustainably and cost-effectively. This collaboration has the potential to create a responsive and inclusive clinical trials system that reflects diverse patient populations and their needs.

Manufacturing Change: Pennsylvania's Efforts to Drive Innovation and Build Biotech Hubs

Alison Beam
Senior Vice President and
Chief Government Affairs Officer, UPMC

Mike Diem, MD
Vice President, Pfizer Ventures

Kaigham "Ken" Gabriel
CEO, Pitt BioForge

Megan Shaw
CEO, Pittsburgh Life Sciences Alliance

Rick Siger
Secretary of Community and Economic Development,
Commonwealth of Pennsylvania

In a discussion centered on Pennsylvania's growing biotech ecosystem, panelists focused on Pennsylvania's potential to leverage its manufacturing heritage and economic diversity to become a leading life sciences hub. The state's top academic institutions — the University of Pittsburgh, Carnegie Mellon University, University of Pennsylvania, and others — drive research and innovation while Pennsylvania's rich centuries-long manufacturing heritage and know-how offer an inherent capacity for large-scale production. Opening remarks by Rick Siger, the Commonwealth of

> **"Pennsylvania's strengths in life sciences are undeniable, but the real question is how we can leverage these to create a thriving biotech ecosystem that benefits the entire state."**

Pennsylvania's Secretary of Community and Economic Development, emphasized how the state's commitment to investing in life sciences is bringing this vision to life. Through initiatives like a recent $600 million economic package dedicated to biomanufacturing and life sciences, Pennsylvania is positioning itself to leverage its broad capacity for academic innovation and practical manufacturing capabilities to transform it into one of the nation's leading life sciences hubs.

Several key themes emerged during the discussion, including the importance of regional collaboration and significant infrastructure investments supporting biotech growth. One example of this is the University of Pittsburgh's BioForge initiative, a project seen as a cornerstone for the continued transformation and growth of Pennsylvania's formidable biotech ecosystem. This initiative will bring advanced manufacturing for complex gene and cellular therapies to the region, enabling faster, more affordable production while ensuring that advanced treatments remain accessible to patients. With its flexible manufacturing capacity and partnerships across research and clinical organizations, Pitt BioForge is on track to accelerate therapeutic development and make southwestern Pennsylvania a central hub for the creation and manufacture of advanced therapeutics.

Workforce development was identified as fundamental to supporting this ecosystem. The panel discussion emphasized that collaboration with educational institutions to build specialized training programs in biomanufacturing and life sciences will ensure a pipeline of skilled workers prepared to drive this sector forward. Furthermore, creating incubation spaces and increasing venture capital investment were discussed as other areas for creating a supportive environment for biotech startups and encourage sustainable innovation. These resources will help Pennsylvania attract and retain talent and businesses, further cementing its reputation as a leader in life sciences.

Pennsylvania's commitment to becoming a life sciences leader represents a once-in-a-generation opportunity for both the state and the region. The discussion concluded with a call for expanded public-private partnerships to create a collaborative investment framework. Aligning government resources with private sector expertise and funding can maximize the impact of investments in biotechnology. Establishing a dedicated, cross-sector investment initiative will enable stakeholders to capitalize on this historic moment, ensuring that the state's life sciences sector continues to grow and lead in the development of transformative health care solutions.

Keynote Fireside: The Evolving Health Care Landscape

Feby Abraham, PhD
Executive Vice President and Chief Strategy Officer, Memorial Hermann Health System

James Bauersmith
Vice President of Translational Sciences, UPMC Enterprises

Diane Holder
Executive Vice President, UPMC; President, UPMC Insurance Services Division

Deneen Vojta, MD
President, Arctos Foundation

A keynote discussion with key health system leaders focused on the escalating costs of novel therapeutics and the need for a sustainable reimbursement model to address these and other rapidly rising expenses of health care in the U.S. The current inefficiencies within our system, where high spending only sometimes correlates with better outcomes, must change. To accomplish this, a shift must occur from volume-based reimbursement models to value-based care, where payment is linked to patient outcomes rather than the number of services provided.

The panel also discussed the need to align incentives across health care stakeholders, highlighting how fragmentation in the system can hinder efforts to improve patient care. Moving from a reactive

> **"We have to become better stewards of health care. It's not just about hospitals or payers — it's about bringing pharmaceuticals, behavioral health, prevention, and genomics together to address total health."**

health care model — focused on treating disease — toward a proactive life care model requires restructuring to promote collaboration and long-term thinking. Such an approach would integrate preventive care, digital health solutions, and at-home monitoring technologies, helping reduce reliance on high-cost interventions while improving patient outcomes.

An additional focus was placed on managing the financial risks of expensive therapies through innovative mechanisms, such as national high-risk pools or a public utility model. These structures would ensure equitable access to life-changing and life-saving therapies, distributing their costs across a broader risk pool which would help reduce the financial burden on individual health plans or payers.

The discussion concluded with a call to action for industry stakeholders to accelerate pilot programs that demonstrate the value of new technologies and modes of care. Breaking through the annual budget cycles of health plans requires more agile collaboration and implementation to leverage the potential benefits of innovative treatments and tools in driving systemic change in health care in the U.S.

Getting Close & Personal II: Improving Patient Care Through Novel Biomarker Discovery

Tim Billiar, MD
Executive Vice President and Chief Scientific Officer, UPMC

Sonja Hess, PhD
Senior Director, Centre of Genomics Research, Discovery Sciences, R&D BioPharmaceuticals, AstraZeneca

Sean Hofherr, PhD
Chief of Clinical Strategy and Product Development, Broad Clinical Labs

Tarek Samad, PhD
Head of Global Research and Corporate Patent & Trademark, Lundbeck

Biomarker discovery and its potential to transform patient care are advancing rapidly. Science and medicine are extending the application of precision medicine into all facets of health care starting with disease diagnosis and patient stratification and extending all the way into treatment response monitoring and surveillance. As the search for biomarkers evolves, they are becoming critical in stratifying patients with multi-factorial diseases, enhancing both drug development and clinical care. The session featured experts who discussed the opportunities and challenges in

“It’s very hard to envision modern drug discovery without biomarkers. They help us move from the phenomenology of a disease to a mechanistic understanding of the pathophysiology at play, which allows us to isolate subpopulations and predict therapeutic outcomes.”

multi-modal biomarker discovery and the potential impact of spatial omics in understanding complex biological processes.

The integration of various omics technologies — genomics, proteomics, and metabolomics — can enable a more comprehensive understanding of disease mechanisms. The panel highlighted how these tools allow for better patient segmentation and more precise targeting of therapies. For example, the use of multi-omics approaches in diseases like Alzheimer's and Parkinson's, where traditional diagnostic methods have been limited, is becoming more prevalent. By combining data from multiple biological systems, researchers can now identify biomarkers that signal disease onset and predict patient responses to specific therapies or likely trajectories for disease progression or severity.

Successful biomarker discovery requires large datasets and the need for cross-institutional collaboration in accumulating and analyzing this data. Also emerging is the role of spatial omics, a technology that enables researchers to visualize the organization of cells within their spatial context in tissues. This approach has the ability to uncover new layers of biological complexity and create new insights into disease progression, which was not previously possible. By mapping how cells interact within tissues, spatial omics can identify new therapeutic targets, leading to more personalized treatments.

The conversation also touched on the regulatory challenges associated with biomarker discovery, particularly the difficulties in securing FDA approval for new biomarkers. While embracing the use of biomarkers is necessary to fully advance the promise and utility of precision medicine, current regulatory pathways remain complex, particularly when biomarkers are used as surrogate endpoints in clinical trials. As regulatory bodies increasingly recognize the clinical value of biomarkers, health care organizations must be ready to navigate the complexities of integrating these clinical tools into routine practice. Successfully implementing biomarkers at the clinical level means that organizations must develop the required infrastructure, clinician training, and standardized protocols for how and when biomarkers are used in screening, diagnosis, and care. Preparing in advance for these shifts is needed for translating biomarker research into actionable patient care improvements and accelerating their widespread or universal clinical application.

"The biggest challenge in biomarker research is the data — gathering, standardizing, and analyzing massive amounts of data. AI and machine learning tools are helping us sift through this mountain of data to pull out useful information, but it's a multidimensional problem that requires innovation at every step."

Mind the Gaps: Driving Women's Health Equity Through R&D, Investment, and Delivery of Care

Robert Edwards, MD
Chair, Department of Obstetrics, Gynecology and Reproductive Sciences, University of Pittsburgh

Caroline Gaynor
Partner, Lightstone Ventures

Ketki Karanam, PhD
Vice President, UPMC Enterprises

Amy Meister, DO
President, UPMC Community and Ambulatory Services

Sandra Milligan, MD
President of Clinical and Commercial Operations, Aspira Women's Health

This panel discussion brought attendees and speakers from the previous day's Women's Health @ Top of Mind symposium to give a recap of the main themes and discussion points from the day. Pointedly: healthier women equate to healthier societies. Simple to understand yet profound in its gravity and implications. The panelists continued the prior day's discussions on the significant gender inequities in health care, the gaps in research, funding, investment, and access to care that women continue to face — and what these translate to in real-world measures.

> **"We need to invest more in research on female-specific diseases like endometriosis and menopause. These are areas that have been historically underfunded and overlooked, but they are crucial to improving women's health."**

The discussion emphasized that while significant research exists on conditions such as cardiovascular disease, Alzheimer's disease, and autoimmune disorders, gender differences within these diseases have historically been overlooked and understudied, to the detriment of women and others. This gap has resulted in an incomplete understanding of how these conditions present and progress differently in women compared to men. Without incorporating gender as a variable, the unique biological and clinical manifestations of disease in women have often gone unrecognized, contributing to less effective treatment options and poorer health outcomes. It should be obvious, but if one primarily views a disease, condition, or treatment through the narrow lens of male biology and applies that universally, the ability to grasp how diseases or treatments affect non-males is essentially obliterated..

Adding to this challenge is the limited engagement, education, and awareness surrounding women's unique health risks at different stages of their lives. A concerted effort to improve awareness among women and their health care providers must occur to ensure that gender-specific health issues are fully understood and addressed.

Another key takeaway from the session was the urgent need for more basic research into female-specific diseases and the biological differences between men and women in disease manifestation and progression. The panel also stressed the importance of more direct investment in women's health, reiterating the striking statistic that only 2% of National Institutes of Health funding is directed specifically toward diseases affecting women disproportionally. This lack of targeted funding perpetuates knowledge gaps, making it difficult to develop treatments that effectively address the unique needs of women.

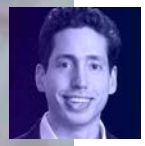
The discussion also covered tangible solutions, including increasing women's participation in clinical trials by making trials more accessible and ensuring that they reflect the realities of women's lives, such as caregiving responsibilities. There was a call for health care providers and systems to adopt value-based care models that account for the unique health care needs of women, focusing not just on reproductive health but also on the prevalent conditions where women's outcomes have historically been poorer than men's.

At a fundamental level, these and other persistent inequities in women's health care and research require multifaceted action, including better research funding, more inclusive clinical trials, and rethinking health care delivery models to ensure that women receive equitable care. This shift is paramount not just for improving women's health but also for creating broader societal and economic benefits.

Hiding in Plain Sight: Can AI Generate Real-World Insights to Solve Big Problems?

Sunita Badola
Senior Director, Head of Oncology Data Strategy and Partnerships, Takeda

Eli Casdin
Chief Investment Officer and Founder, Casdin Capital



Andrew Epstein
Special Counsel, Cooley

Rob Hartman, PhD
Senior Director, Translational Sciences, UPMC Enterprises

Milind Kamkolkar
Venture Partner, RA Capital Management

Oscar Marroquin, MD
Executive Vice President, UPMC; President, UPMC Physician Services

There is a vast, largely untapped potential for AI to harness the wealth of data stored in electronic health records (EHRs) for drug discovery and patient care improvements. Health systems generate enormous amounts of clinical data, and by leveraging AI, they could uncover new uses for existing drugs and identify novel therapeutic targets. However, fully realizing AI's potential in health care requires addressing significant challenges, particularly in establishing safe and ethical data sharing practices. Effective use of AI will depend on developing robust data infrastructure, standardization, and secure tools to facilitate collaboration across systems and providers. By ensuring that data can be shared responsibly and analyzed in real-time, the advancements and insights that AI can help to generate will power greater utility from the data that has been and will continue to amass.

> **"The ultimate value of AI in health care lies in its ability to transform data into actionable clinical insights, but the challenge is in ensuring the data we collect is consistent and standardized."**

While AI has the inherent potential to transform health care by mining real-world data in real-time for insights, the current structure of most health care systems makes this exceedingly difficult. Data is often siloed within different departments and institutions, making it hard to conduct large-scale analyses. Data fragmentation and the lack of standardization across health care systems are barriers to effective use of AI tools and techniques in generating actionable insights from raw clinical metrics. In addition, most data collected within EHRs was initially designed for something other than research purposes. This further complicates efforts to extract useful information for things like drug target development or clinical decision-making.

Recruiting patients remains one of the most persistent challenges in clinical trials, with approximately 80% of trials falling short of their enrollment targets. Lack of enrollment leads to delays and compromises the quality of the resulting data. This dynamic points to the need for systemic change in how participants are identified and engaged. AI, combined with patient data, could help solve some of these challenges by identifying patients who are more likely to respond to a specific treatment based on known, modeled clinical data points. An AI-driven approach could significantly reduce recruitment timelines and trial costs by narrowing the focus on whom to enroll. However, to achieve this, better data governance and cross-institutional collaboration are needed to facilitate the sharing of high-quality data that can feed AI-powered decision-making algorithms. If you're looking for a needle in a haystack, one approach is to make the haystack smaller at the beginning.

Also important is creating multidisciplinary teams that include clinicians, data scientists, and engineers to drive these innovations forward. Simply having the data is not enough. The data must be clean, well-curated, and, most importantly, aligned with clinical realities. Achieving this requires a robust partnership between the health care industry and tech companies to develop tools that can accurately process and analyze the mountains of data without compromising patient privacy or data integrity.

There also was a discussion about the role government regulation and incentivization play in improving data sharing across systems or universalizing the data. Governmental or policy support is likely needed to create frameworks for nonexclusive data licenses that allow health care systems and industry partners to collaborate more freely without the traditional financial and bureaucratic barriers that can blunt access to data or more meaningful use of the data that already exists.

Not all of this has to be completed or achieved simultaneously or immediately. Incremental progress in leveraging AI for real-world insights is likely the best path forward. While the goal is to have large, scalable AI models capable of providing groundbreaking insights with a few keystrokes, the health care industry needs a short-term focus on smaller, more manageable improvements to build trust and acceptance of the technology. For example, AI could be used in the short-term to assist clinicians with triaging more routine decisions, freeing them up to focus on more complex case management. This kind of stepwise approach for building the infrastructure and partnerships needed to fully realize AI's potential in health care is a realistic and achievable goal.

Partnering for Change: Collaboration is Key to Develop Novel, Complex Tx Modalities

Leah Byrne, PhD
Founder and Chief Scientific Officer,
Avista Therapeutics

Frances L. Johnson, MD
Chief Innovation Officer,
Perspective Therapeutics

Keir Loiacono
CEO, BlueSphere Bio

Etienne Montagut
President, PharmaLogic

Mike Paglia
Chief Technology Officer, ElevateBio

Don Yealy, MD
Chief Medical Officer, UPMC

Highlighting the transformative potential of advanced therapies such as gene and cell therapy and radiopharmaceuticals, this session also addressed the complexities of developing and administering these often highly individualized, complex agents that require special handling and time-sensitive supply chains. These emerging modalities offer significant promise for patients, but they also present challenges related to their manufacture and distribution, supply chain logistics, the availability

“Gene therapy, cell therapy, and radiopharmaceuticals offer immense promise but come with unique challenges. We need strong partnerships to navigate these complexities and ensure patients have access to these innovations.”

of components, and clinical testing. As these therapies move from experimental phases to broader clinical use, UPMC's proactive investments and partnerships with industry and academic partners play a critical role in bridging the gaps and accelerating the journey from discovery to patient access.

UPMC and the University of Pittsburgh's joint efforts in cell, gene, and radioligand therapy illustrate this commitment. By collaborating with innovators and sharing the inherent risks of developing these unproven modalities, UPMC is strategically removing some of the risks on the path from experimental therapy to approved treatment. This approach — the UPMC Enterprises approach — is grounded in visionary investment and proactive partnering, enabling a more streamlined and resilient framework that can bring therapies to market faster but also ensures their accessibility and scalability. This approach to partnerships creates a new standard for risk-sharing and long-term investment in health care solutions that can fundamentally change how patient care is provided.

One way these modalities can more easily reach their intended targets is through distributed manufacturing systems, where production occurs closer to patients and treatment sites. This approach minimizes logistical challenges, particularly for time-sensitive therapies like radio-pharmaceuticals, which have short half-lives and require near-immediate administration. By establishing multiple localized facilities, distributed manufacturing not only speeds up delivery but also creates valuable redundancies that strengthen the supply chains responsible for providing treatments. In the event of demand surges, logistical disruptions, or facility-specific delays, other sites can seamlessly continue production, ensuring continuous availability. This type of flexible, decentralized model makes it possible to scale the creation and use of advanced therapies, improving patient access to life-saving treatments while enhancing the resilience of health care delivery.

The discussion also addressed the ongoing need for early collaboration between drug developers and manufacturers. By engaging with manufacturing partners at the outset of the drug development process, companies can more effectively avoid costly delays and ensure that clinical trials using these agents stay on track and are not compromised by insufficient levels of the therapeutics being tested.

About the Center for Connected Medicine

The Center for Connected Medicine (CCM) at UPMC is defining the future of the modern health system through programming that informs, connects, and inspires leaders and innovators in health care. Collaborating with a network of experts from across the health care ecosystem, the CCM focuses its research and events on consumer-centered solutions, digital transformation, and scientific and medical innovation.

Center for Connected Medicine

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About UPMC Enterprises

UPMC Enterprises is the innovation, commercialization, and venture capital arm of UPMC, a world-renowned, nonprofit health care provider and insurer committed to delivering exceptional people-centered care and community services. Working in close collaboration with innovators from UPMC and the University of Pittsburgh Schools of the Health Sciences, as well as others worldwide, UPMC Enterprises accelerates science from the bench to the patient's bedside. With an emphasis on translational sciences and digital solutions, UPMC Enterprises provides its diverse portfolio companies and partners with capital, connections, and resources to develop solutions to health care's most complex problems.

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