**Schedule subject to change*

Preliminary Agenda

Monday, September 9, 2024

11:00 am – 3:00 pm	**Pre-Event Networking Excursion & Lunch** Join us for a visit to the National Museum of African American Music, including a delightful lunch. Get the networking started early while immersing yourself in a local pride of Nashville, the only museum dedicated to the history of Black music, integrating history and interactive technology to bring musical heroes of the past to the present. Discover the stories behind the rhythms and melodies that have shaped genres from jazz and blues to hip-hop and R&B. **Limited Capacity, additional registration required. Email: conferences@biocentury.com for more information**
4:00 pm – 4:30 pm	General Registration & Networking
4:30 pm – 5:30 pm	**Opening Plenary & Fireside Chat: Heads of Pharma Research, in Conversation**
5:30 pm – 7:00 pm	Networking Welcome Reception

Tuesday, September 10, 2024

7:30 am – 8:45 am	**General Registration & Networking Breakfast**
8:00 am – 5:30 pm	C-level Partnering Meetings
8:45 am – 9:00 am	Official Welcome from BioCentury
9:00 am – 5:30 pm	Presenting Companies
9:00 am – 10:00 am	**McKinsey & Company Keynote: Grand Rounds Conference Report**

Grand Rounds I: THE BOTTLENECKS

Uncorking innovation: Critical issues hampering the acceleration of translation

Against the backdrop of escalating discoveries and breakthroughs over the past decade, critical bottlenecks are holding back their conversion to value-driving drug development. Inefficiencies in translation extend from poor disease models to underdeveloped delivery technology to mismatched expectations for what constitutes validation. *The Bottleneck* Rounds will unpack the strategies for overcoming these roadblocks.

10:00 am – 10:40 am	**From causal biology to preclinical validation: What makes the grade in 2024?** Scientist-entrepreneurs hope to win attention and money from investors and pharmas, but often run afoul of what evidence constitutes preclinical validation. Drug developers are making clear that causal biology is the North Star, however reaching it is not straightforward. The issue spans the target's origin, model systems, experimental design, statistical robustness and effect size that will convince the buyside a signal is a meaningful result. This Round will discuss what investors and pharmas want to see, and how ambitious scientists can strengthen the case for translating their research projects from academia to industry.
10:40 am – 11:10 am	Morning Networking Break

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11:10 am – 11:50 am **The human first era is here: Is there still value in new animal models?**
Animal models arguably have done what they can for translation. They'll likely persist for PK and tox studies. For functional readouts in disease, however, use of animal models is driven by inertia, routine, regulatory requirements and lack of other options, rather than their predictive potential. With investors and pharmas increasingly seeing human data as ground zero for drug development, where should researchers be focused on building model systems? This Round will discuss what animal models are proving their worth, where transformational new ones might emerge, and what alternative systems, such as patient-derived iPSCs and organoids, are yielding proof of concept that investors, pharmas and regulators will accept.

11:50 am – 12:30 pm **Delivery on demand: Still the elephant in the room**
The rush of new modalities has not been matched by advances in highly targeted delivery, particularly for nucleic acid therapies. Enabling DNA- and RNA-based therapies to reach any cell in the body, on demand, would open untold target space and therapeutic opportunities. Beyond viral vectors, a slow wave in nonviral technologies is building. This Round will evaluate which approaches to tissue-specific delivery promise greater success and consider whether precompetitive approaches or delivery-focused service models will benefit both academic researchers and drug developers.

12:30 pm – 1:30 pm Lunchtime Plenary

Grand Rounds II: THE MODALITY GROUNDBREAKERS

A mountain of modalities: Now comes the translational loop

Academic innovators have powered most of the groundbreaking technologies that have yielded mountains of new modalities from CRISPR to gene therapies to CAR T to mRNA. The handoff to industry is allowing such discoveries to reach patients, but some of these transformations must overcome limitations that cap their use. For these, the translational loop puts the creation of next-generation iterations back in the hands of researchers. *The Modality Groundbreaker* Rounds will identify these potential solutions, and address how drug developers can prioritize among the innovations in a modality to bring treatments to patients most efficiently.

1:30 pm – 2:10 pm **The heyday of AAV engineering is here: Will it deliver?**
AAVs have all but crowded out other viruses as the gene therapy vector of choice, and now promise to go beyond genetic diseases via the creation of vectorized antibodies and proteins that could compete with traditional biologics. Engineering advances with capsid optimization, inducible expression technologies and packaging are solving for toxicities, limits on targeting precision, cargo size, scalability and other manufacturing hurdles. This Round will evaluate the most efficient ways for drug developers to exploit this burst of innovation and consider whether other vectors — viral or non-viral — will see the same kind of progress and diversify the options.

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2:10 PM – 2:50 PM	**The regenerative moment: Are iPSCs poised to reshape the cell therapy toolbox?** With more than 20 types of cell therapies derived from induced pluripotent stem cells in their pipelines, biotechs are on the cusp of breaking open a major new avenue for engineering cell products with a desired set of functions. This Round will ponder important questions that still remain: In the age of single-cell transcriptomics and other deep profiling technologies, what insights can drug developers glean about the fidelity of reprogramming and durability of the cells? What is the field learning about best practices for manipulating and scaling the cells without compromising their benefits? And what cell types are most amenable to replacement by iPSCs?
2:50 pm – 3:20 pm	Afternoon Networking Break
3:20 pm – 4:00 pm	**Maximizing the impact of gene editing: Incremental improvements, or leaps to newer technologies?** CRISPR-Cas9 has catalyzed a world of therapeutic programs, but the fundamental limitations of gene editing still haven't been solved. Researchers have discovered new nucleases yielding base, prime and epigenetic editors that allow pinpoint changes and avoid double-stranded DNA breaks. Next-generation transposases and recombinases that facilitate large-scale genomic alterations are coming up fast. The potential for off-target edits remains, however. This Round will ask whether incremental advances in clinical-stage technologies can remove that barrier, or whether it will take a shift to newer types of biological machinery.
4:00 pm – 4:40 pm	**Souping up cancer cell therapies: Is the juice worth the squeeze?** As cytotoxic cell therapies have carved out their niche, cancer researchers are churning out new strategies to improve their performance, from knocking out genes that promote exhaustion, to adding in stimulators or switches that respectively boost or limit their expansion capacity. What are the limits to the number and types of manipulations these cells can undergo? This Round will identify which CAR T cell manipulation strategies are rising above the noise, particularly for solid tumors, and ask whether other cancer-killing cell types such as TILs and NK cells can benefit as well.
4:40 pm – 5:30 pm	McKinsey & Company Breakout Session
5:30 pm – 7:00 pm	Networking Reception
6:00 pm – 7:00 pm	Poster Spotlight Presentations: At the Forefront of Translational Innovation
7:00 pm – 9:00 pm	Networking Dinner

Wednesday, September 11, 2024

8:00 am – 9:00 am	**General Registration & Networking Breakfast**
8:00 am – 3:30 pm	C-level Partnering Meetings
9:00 am – 12:30 pm	Presenting Companies
9:00 am – 10:00 am	Keynote Address

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Grand Rounds III: THE HUMAN DATA REVOLUTION

The human data revolution: Leveraging academic-industry symbiosis

Academics have largely been the keepers of both the data and the methodological innovations that will advance the data revolution in biomedical science. Industry is critically dependent on applying the best tools to harness these data lakes to uncover causal disease-driving targets, predictive correlates of response and granular understanding of disease subtypes. This marriage of academic science with industry's needs could change the probability of success in discovery R&D by orders of magnitude. But the path is not simple. *The Human Data Revolution* Rounds will identify what must take place to make these data troves fit for purpose.

10:00 am – 10:40 am **Methods-for-purpose: Analytical tools that parse signal from noise, without flattening complexity**
Data lakes are only as useful as the analytical methods that sift through them. The challenge is to mine trillions of data points from omics biobanks and massively parallel functional screens. Long-established analytical methods now sit alongside an explosion in new techniques, including large language models. This Round will discuss which analytical methods are gaining consensus as the right tools for extracting actionable insights and look ahead to the bleeding edge methods that will define the next era.

10:40 am – 11:10 am Morning Networking Break

11:10 am – 11:50 am **Data-for-purpose: Go big, go deep, or go long?**
In an ideal world, researchers would have access to longitudinal high-resolution data on large and diverse human populations. In real life, logistical constraints usually force trade-offs. When is it best to prioritize population size, and when is the depth of data resolution per person more valuable? What questions are only solvable with longitudinal measurements? And will tokenization and data linkage make collection of these high-value data sets more routine? This Round will discuss what leading drug developers, academics and investors view as the optimal paths to making data fit for purpose.

Grand Rounds IV: THE DISEASE BIOLOGY BOOM

While you were away: Strides in disease biology presage a translational boom

Industry's focus on specific diseases has waxed and waned, driven by the commercial landscape. All the while, academic labs have been uncovering new disease biology and sowing the ground for leaps forward in translation. As obesity, inflammation and immunity, and neuroscience come back into favor for pharmas and investors, research labs are offering new targets, mechanisms of action, modalities and strategic approaches to propel those fields into a new age. *The Disease Biology* Rounds will elucidate how emerging science may make drug developers better prepared to serve these large, chronic diseases than the last time around.

11:50 am – 12:30 pm **Unlocking progress in neurology: How to build the case for a new target**
The call to researchers is clear: Deliver new targets and biomarkers that enable industry to reinvent how neurodegenerative and neuropsychiatric diseases are treated. Multi-omics can yield new genetic risk factors and insight into disease mechanisms that can point to new targets, but dismal preclinical models are a roadblock. This Round will explore what it will take to build a strong case for a new target in the era of human data, including where EEG-defined signatures, imaging tools such as FMRI, and clinical and digital phenotyping fit into the picture.

12:30 pm – 1:30 pm Networking Lunch

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1:30 pm – 2:10 pm	**Immunity and inflammation inflection point: What determines the direction from here?** Dramatic improvements in treating autoimmune diseases boil down to a few targets plus successful clinical and commercial strategies. What are the next targets and mechanisms that could allow drug developers to recapitulate their success in new indications and refractory patient populations? Will the early academic data on durable remissions with CD19 CAR T cells have applications in diseases with less B cell involvement? And can bispecifics offer a path to reset the immune system? This Round will probe these developments and ask whether ambition in the field has changed from managing autoimmune diseases to curing them.
2:10 pm -2:50 pm	**Hunger games: The incredible appetite for more innovation in obesity** The earth-shattering success of GLP-1s in obesity has propelled quality and maintenance of weight loss to the top of the to-do list for translational scientists. Can durability and the rebound effect be solved by reprogramming metabolic cells to allow treatment breaks? Which targets and pathways might shift the type of weight loss to preserve muscle? What role might AI and data science play in identifying precision targets? Is prevention of obesity a realistic goal? This Round will handicap progress on these questions and consider how the effects of GLP-1s on liver inflammation, renal failure and other systems extend the benefits into other major disease areas.
2:50 pm – 3:30 pm	Closing Rapporteur Plenary
3:30 pm – 4:30 pm	Closing Networking Reception
4:30 pm – 6:00 pm	Local Ecosystem Tour **Limited Capacity, additional registration required. Email: conferences@biocentury.com for more information**