

NORTH AMERICA *DIA 2014 50TH ANNUAL MEETING*

Q&A with *DIA 2014 50th Annual Meeting* Keynote Speaker Jamie Heywood



On Monday June 16, Jamie Heywood will deliver the Keynote Address at our *DIA 2014 50th Annual Meeting: Celebrate the Past, Invent the Future* in San Diego, CA. Jamie is the Founding Director of the ALS (amyotrophic lateral sclerosis) Therapy Development Institute, the world's first nonprofit biotechnology company, and Co-founder and Chairman of PatientsLikeMe, an innovative web community that allows patients to pool their disease and treatment experiences to advance medicine.

"Each of us has someone we love who has been affected by what we do in the life sciences industry. For me, that person was my younger brother Steven, who was diagnosed with ALS at the age of 29 in 1998. I began by founding the ALS Therapy Development Institute, the world's first non-profit biotechnology company, to directly develop treatments for Stephen and all ALS patients," he explains. "In addition, my brother Ben, our friend Jeff Cole and myself founded PatientsLikeMe,

a social network that allows to use rigorous health outcome measures to understand their own disease, inform the development of treatments and improve health care. We currently have more than 250,000 members sharing information on more than 2,000 conditions."

"This year, I'm honored to be the Keynote Speaker at the *DIA 2014 50th Annual Meeting*. We will *Celebrate the Past and we will Invent the Future*. That future, I believe, will be one where patients are our partners – where industry and patients meet as equals using quantified data from patients' real-world experiences to inform every part of the decision about how to develop and deliver a drug or healthcare service to the market. I am passionate about this vision and I'm excited to share it with you in my keynote presentation on Monday, June 16, in San Diego at the *DIA 2014 50th Annual Meeting*. So please join us, and let's build this future together."

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What does the term innovation mean to you and would you provide an example of how you have observed or applied this meaning in your own work?

My take is that innovation, especially in health care and drug discovery, means looking beyond where the system is currently. It's about going back to the driving principles and asking, "Why was the system designed to do this in the first place?" Let's look at drug discovery, for instance.

Clinical trials are generally the only accepted evidence model – and we do them according to a specific protocol and standard. While there is much innovation on how to do them, I think more disruptive innovation occurs when we ask, "Why do we do them?" The goal is to generate actionable evidence that can benefit patients, clinicians, payers and regulators. We all know many of the faults of today's trials, yet we often reject any other approach to collecting evidence that might meet our original goal. In this case, innovation is asking, "Do we have to do clinical trials to get those answers?" You have to ask that sort of fundamental, upward question and think about how to solve that problem, but this kind of transformative innovation is not widely embraced by industry today. If you really ask "Why," then you end up with potentially much more transformative solutions.

Innovation should not be reckless, though. You have to go through the process of determining whether your innovations are practical or meaningful, or meet the needs of the current system or how they could evolve to do that. It takes real investment to learn. At some level, I view innovation,

particularly in health care and drug discovery, as being driven by the people we are trying to serve: The individual or patient who we are trying to help – whether it's with a drug or a change in the way that health care is delivered or a service that impacts some other part of their lives. The real driving question is: What do they really need?

A friend of mine used to run a consulting firm that specialized in customer service, and an airline hired him to find out what their customers cared about. He did it in a novel way – he just asked people. (This was about thirty years ago, before that was "normally" done.) He reported back to the airline that what your customers care about the most is parking. The airline said, "But we're not in the parking business." And my friend replied, "Well, your customers think you are!"

If you ask your customer, you will often learn where your assumptions are inaccurate. That's powerful information to have when trying to illuminate innovation opportunities and ways of making the system behave better or operate better. This is largely underestimated in its value, particularly in the drug industry. One reason is that it's very hard for pharma to innovate. The industry operates under very rigorous rules because small errors can be hugely consequential and expensive. If I owned a drug company, I might also be hesitant to let my directors innovate because the costs of failure are so expensive. It's a particularly challenged industry because of all its regulation.

But, on the other hand, we all know the system *has* to do

better because patients need it to. That's part of the value that PatientsLikeMe tries to bring to bear – we can both define and understand patient needs while also knowing and respecting the rules that the industry has to operate under to demonstrate and deliver value and meet their regulatory and legal requirements. Pharma companies know where they want to go and that they need to be closer to the people using their products; they just don't necessarily know how to get there. Finding that path, and navigating all of its challenges, is the hardest part of being innovative and it is what's required for industry to more effectively meet patient needs.

May we ask you to further explain "using quantified data from their [patients'] real-world experience to inform every part of the decision about how to develop and deliver a drug to the market"?

For the past ten years, we've been collecting data – structured and clinically relevant data – directly from patients who are all willing to share it through this online network. I'm talking about using those real-world experiences to inform and improve the drug discovery process.

And when I say that, I mean *every part* of drug discovery. You can use this information to help identify which emerging research might create significant value, or identify whether the initial findings are meaningful or worthwhile. You can use this information to identify what measures would be most effective in trials. You can use this information to inform what tolerability or safety attributes



will make your drug “worth it” to patients, or use the information to decide whether you can achieve meaningful clinical efficacy. Patients’ real-world experiences should directly inform ALL these things.

When you bring patients in as partners to the process, there’s so much that can be improved. You can answer questions like: Where’s the best place and what’s the best way to run this trial? What things would make your trial easier to enroll? Where can you get the most signal from patient experience? Which patients should you target and how do you stratify them into the right populations? Their input can help with the pre-launch process of identifying what the market’s going to be for your company. It can also help you can do risk management *after* the drug is released by measuring whether there are things you didn’t see in the trial that would help them or hurt them.

Industry does some of this today through market research, through epidemiology, through data mining or through running clinical trials and building registries. But I think we can push the boundaries and, frankly, operate in a more patient-centered manner. I’m proposing that a partnership between drug companies and individuals – one built around patients’ real-world data and experiences – can answer many of those above questions in more cost-effective way than we do it today. And, in some contexts, it may produce data that is greater in richness and value than what we’re collecting through our “standard” methods. This partnership can make the process faster, richer and better,

with the added benefit that it provides a self sustaining system to empower patients to continue to improve the entire system on their own.

Launching a successful drug is the sum of a thousand good decisions. Increasingly, the difference between a successful blockbuster and something that fails will be the ability to bring the patient’s experience into the decision-making process, in a way that is time and cost-effective and that accelerates the discovery process. This increases the probability that those decisions will be the right ones. PatientsLikeMe is trying to figure out a new model where these partnerships deliver better insights and answers to these questions, and also creates value for the patient that lasts beyond each single discovery exercise.

Good companies today are learning to do this well in many ways. In the health care system, the drug industry is among the entities most aligned with what patients are looking for: Finding new innovations that change and improve their lives. This is really important: One of the reasons that PatientsLikeMe works with the drug industry is because we believe that they are largely the part of the health care system most aligned with patient objectives – to understand how to get to the best outcome they can achieve. The drug industry usually gets paid when that alignment is successful. Of course there are some caveats and it’s not a perfect system, but at the moment, it’s the best we have. The question: How can we make it better?

What are your thoughts on the patient-focused components of the FDA Safety & Innovation Act?

My read is that, in general, things seem to be going in the right direction. The FDA is requiring increased input from patients and increased measurement and demonstration of real world clinical value. There are some thoughtful ideas from the FDA on how to do that well. As a government agency, I have to give it pretty good marks for engaging well in dialogue and learning about where the policy and the science and the people are going. On an operational level though, my sense is that the FDA is a little bit of a slow bureaucracy. There’s the problem where the leadership may want something and be moving in the right direction, but the troops are still moving in a different direction. If I were in leadership at a drug company, I would be very conservative in the way I adopted some of the FDA’s more leading-edge guidelines because, in my experience, you have to strike a balance between following the aspirational objectives of the new guidelines and the reality of the “feet on the ground” operational interpretation of the committees, inspectors and operational staff who make the decisions.

I want to mention an area that I think is really broken and where I think there is opportunity for massive improvement. If one were trying to design a system to understand and learn about the real-world risks and benefits of treatments after approval that would expend the *most amount of money* in the *least effective way*, I think we’d be hard-pressed to design something worse than



the one we have now. I believe the post market surveillance model (as it exists today) creates a massive “waste tax” on the drug industry with incentives that discourage organizations from learning effectively in the real world about the benefits and risks of drugs. More importantly, it’s a system that provides very little actionable information to patients or clinicians. Regulators and companies play games of “gotcha” centered around poorly-measured, spontaneous events that rarely deliver any true knowledge about the risks or benefits of a drug...all while adding up to billions in costs. This desperately needs to be revisited and it needs to be revisited from a patient perspective: How can you as a patient trust that everyone in this system is working in your interest, in a way that produces the best information? If we design a solution for that kind of innovation and discovery, then I think there is a chance to advance toward a learning system that serves patients, clinicians and regulators while eliminating huge waste.

Many DIA attendees, members and volunteers come from or work with regulatory agencies. What message do you hope your keynote address delivers to these regulatory professionals?

I think that we’re standing at the transition point to a new era, not unlike an earlier parallel transition from the computer industry. There was a time when mainframe computers dominated the day – back when personal computers

were just starting and “weren’t relevant.” The only way to do computing research was to be within a big university or company with a big budget and a large team. That context really limited who was able to ask and answer the questions and who could use the resources that allowed them to be able to do the research.

What’s happening right now is the beginning of what I call a “personal discovery phase” where individuals are connecting on networks and beginning to do their own research and develop their own understanding. Right now, it’s kind of baby or novel research with early proof points. Again, it’s a little bit like the early personal computer space, where the mainframe guys were saying, “That’s cute. It’s nice that you can write your little games with these computers, but they’re never going to really matter.” I think we’re at that moment in the translation of biomedical research. The mainframe research systems in academia and the drug industry are about to learn that the distributed human network can deliver really interesting value and, in the end, perhaps eclipse them.

So my message to the FDA and to industry is to some degree: Don’t make the mistake of underestimating the power of this emerging patient-research network in the same way that the original mainframe computing industry underestimated the impact of the emerging personal computer network. Some of the science I’m going to illustrate in my keynote presentation only works under selected conditions – and there are still many issues about bias, models, and methodologies that aren’t worked out. But from a regulatory

standpoint or from a discovery standpoint, if your eyes are only focused on what you consider to be the validated traditional approaches, you will suddenly find that the world is listening to something else and discovering something else without you.

As it was during the HIV crisis to AIDS activists, my sense is that the FDA can be responsive when pushed. Theirs are aligned missions – patients generally want the same thing that the FDA wants. But I think we’ll find that the emerging patient and advocate communities are going to invent methodologies and approaches that will eclipse our current fixed thinking. When the shared truth about the effectiveness of drugs comes into the world through a new methodology...if you’re not ready to talk about it, if you haven’t looked at it and you haven’t been thinking about it, then you won’t know how to regulate it and you’ll be in danger of losing your relevance. I say let’s all pay very close attention to where the advocates, the data and the patients are going. The people who think, “Oh, these patients – they don’t really understand research, they don’t really understand statistics, they don’t really understand how to do this stuff,” are going to be very surprised at how quickly Moore’s Law changes the rules in biology and medicine the same way that it did in computing.

I am really excited about this discussion of the future. I think it’s a really important forum that DIA is working to create. The chance to create human good is amazing. ●