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**the
Medicine Maker
100
Power List
2017**

Meet the Top 100 most
inspirational professionals
shaping the future of
drug development.

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Online this Month



Sticking to Your Stent

For drug eluting stents to work, the patient must take their medication for 12 months – and non-adherence can increase the risk of morbidity. Andrew Boyd and his team at the University of Illinois have developed a new app to help improve clinical outcomes. <http://tmm.txp.to/0417/stent>

The Humanity in Science Award

Entries for the Humanity in Science Award will close on May 1, 2017. Time is short but if you miss out then keep your eyes on www.humanityinscienceaward.com for entry details for 2018.



Bringing API Manufacture into Focus

There is a shift in the industry towards biopharmaceuticals, but there is still great

demand for synthetic APIs. We catch up with Richard Hercek and Boris Petrussek from Saneca Pharma to discuss the latest trends in small-molecule API manufacture.

<http://tmm.txp.to/0417/hercek>



The Power List

You'll find our annual Power List on page 22, but don't forget that you can

also access the Power List online at:

<https://themedicinemaker.com/power-list/2017/>



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Pharma Party,
by Stephanie Sutton

On The Cover



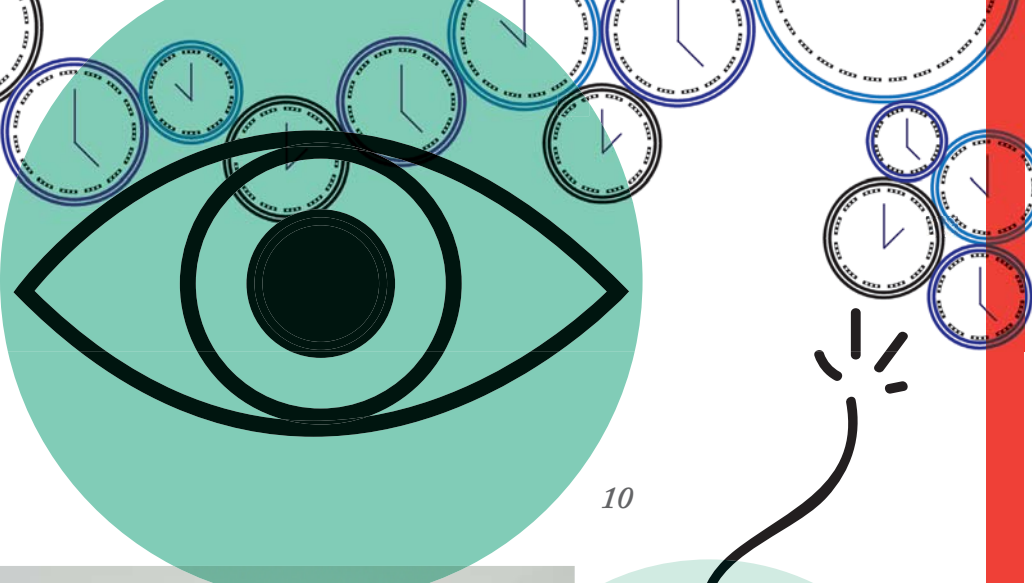
What better way to highlight the vibrant stars of drug development than with the striking artwork of Piet Mondrian?

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For the third year running, our Power List celebrates the top 100 most influential people in drug development, including masters of the bench, industry influencers, business captains and champions of change.

the Medicine Maker

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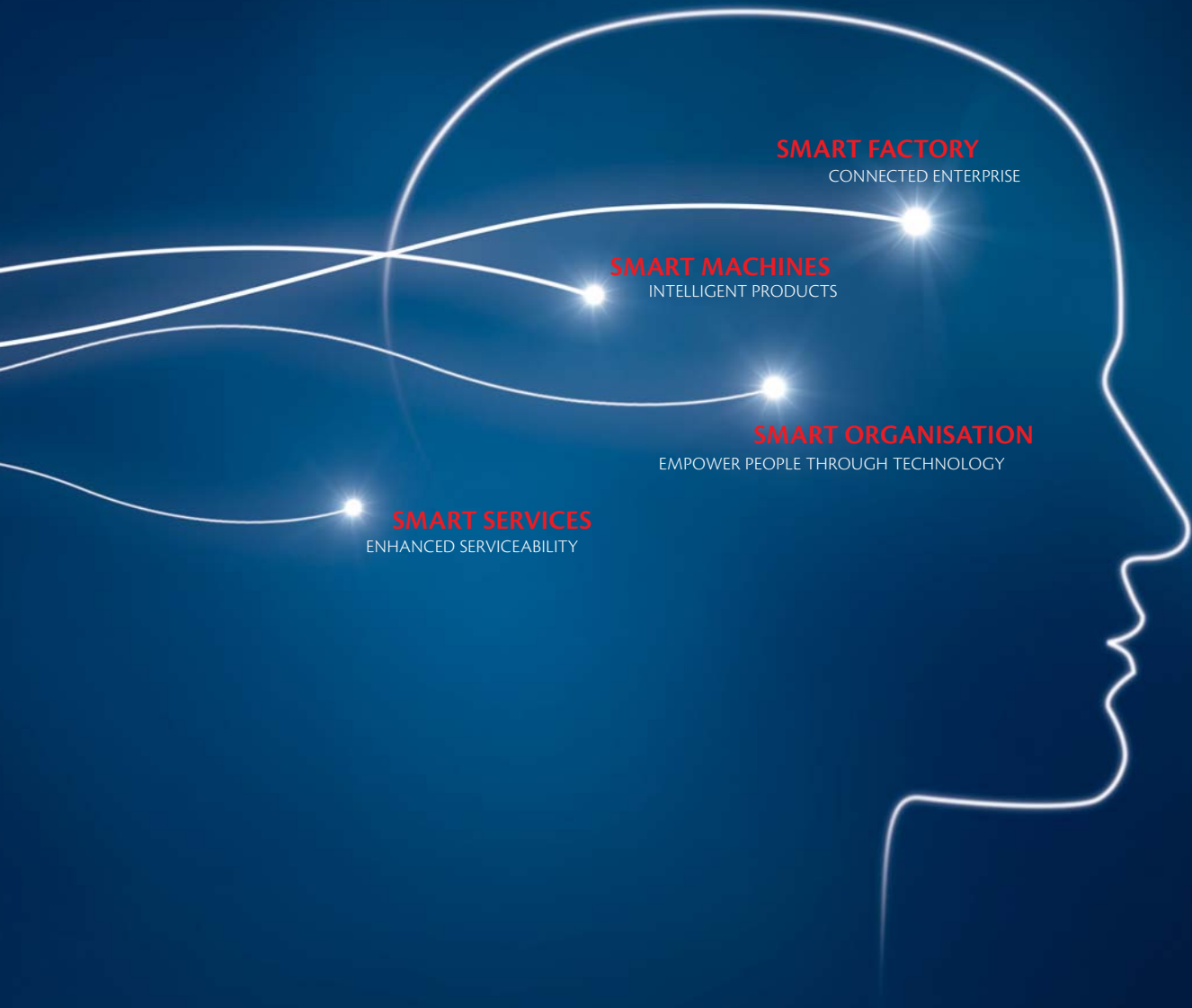
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The Medicine Maker was launched with a clear aim: to celebrate the people, processes and vision that bring new drugs and biologics to market. It's fair to say that the pharma industry is not always looked upon favorably by the public – and though drug pricing, side effects, product recalls and corporate malpractice are topics that must never be ignored, it's important that they do not overshadow the achievements, success stories and passion of the people who work in the industry. And so, The Medicine Maker Power List hails a hundred of the best and brightest individuals involved in bringing new drugs to patients, as nominated by readers and selected by an expert judging panel.

Our previous Power Lists, published in 2015 and 2016, each ignited much discussion – and a fair amount of controversy. For example, Mylan's Heather Bresch topped the list in 2016, but has since been embroiled in a number of high-profile pricing arguments. Did she make the list in 2017? Turn to page 22 to find out!

No list can (or should) be definitive, but we do hope that our Power List shines a spotlight on the wonderful variety of talent that goes into bringing a medicine from bench to bedside. The 2017 Power List also brings with it a slight twist that recognizes an increasing number of nominees and their diverse backgrounds and expertise; drawing comparisons is challenging to say the least. To this end, we have divided the 2017 Power List into four categories:

- Masters of the Bench (page 24) – scientists and researchers whose late-night lab work lays the foundations for new therapeutics.
- Industry Influencers (page 31) – individuals who drive industry best practices and regulations, as well as new manufacturing techniques and technologies.
- Business Captains (page 37) – business leaders and entrepreneurs that turn scientific ideas into marketable medicines.
- Champions of Change (page 43) – patrons of the industry who are striving to make the world a better place by getting medicines to those who need them the most.

I hope you will all join me in offering a very warm congratulations to everyone who made the list. But if you don't agree with the results (which is part of the fun), remember that the Power List will be back again in April 2018 – and nominations for the 2018 list are already open at: <http://tmm.txp.to/2018/powerlist>.

Stephanie Sutton
Editor

Stephanie Sutton

Upfront

Reporting on research, personalities, policies and partnerships that are shaping pharmaceutical development and manufacture.

We welcome information on any developments in the industry that have really caught your eye, in a good or bad way. Email: stephanie.sutton@texerepublishing.com



Medical Miners

Intrepid researchers scour disused mines in search of new biochemistries for drug development

The number of active coalmines is diminishing, but older mines may still have their uses. Specialists in “bioprospecting” – the search for new organisms and biochemistries that could be useful for making drugs – say they have made new versions of the antibiotic daptomycin, using a prenylating enzyme obtained from the smoke vents of a coalmine in Kentucky, USA. The enzyme – named PriB – was isolated from a soil bacterium and could be a useful biocatalysts for drug developers. Prenylating enzymes have been used to modify simple molecules before, but the benefit of PriB is that it can modify more complex drugs – like daptomycin

We caught up with Jon Thorson, Director of the University of Kentucky’s

Center for Pharmaceutical Research and Innovation (CPRI) and co-author of the study (1), to find out more about “bioprospecting.”

How did you get involved with this field of research?

A central theme in our research is the discovery and synthetic application of new enzyme-catalyzed transformations, with a particular emphasis on the biosynthetic pathways used by nature. A natural extension was to get involved in the actual natural product discovery process – so we launched an initiative as part of the new UK CPRI. Though the discovery of new bioactive natural products is a key deliverable of a program like ours, the effort also exposes potentially useful new biocatalysts and biosynthetic pathways. Coalmines and other subterranean environments are a great new place to look for unique biodiversity.

What exactly is PriB?

PriB is a highly permissive indole C-prenyltransferase, the gene for which

was found by sequencing the genome of a microbe isolated from the Ruth Mullins underground coalmine and seam fire site. We previously reported that this particular microbe produces a series of new metabolites that contain a uniquely functionalized carbohydrate as part of their structures (2). Biochemical characterization of PriB revealed unusual permissivity in terms of the enzyme's ability to use both a wide range of non-native prenyl acceptors and donors as substrates, including the complex natural product-based antibiotic daptomycin. These modifications were subsequently found to improve daptomycin's antibacterial potency in vitro. The structure of PriB, determined by George Phillips Jr. and colleagues, presents a structural blueprint for understanding PriB ligand recognition and catalysis

– and could potentially guide future catalyst engineering. Shanteri Singh also pioneered the surprising discovery that other previously characterized prenyltransferases could also modify daptomycin.

What does the research mean for drug companies?

Though prenylation improves the antibacterial potency of daptomycin, the impact on fundamental drug properties and in vivo efficacy remain to be determined. However, our work offers new enabling biocatalysts and corresponding genes for natural product analoging (via biochemical or strain engineering approaches) that could extend to a variety of drugs beyond daptomycin. Our CPRI natural product repository now contains more than 1000 distinct microbial strains

and more than 300 pure microbial natural products (50 percent of which are exclusive to our collection) as a potential source for new industrial biocatalysts and/or new lead compounds. As a related example of biocatalyst discovery and development, our longstanding effort in small molecule glycosylation contributed to the discovery of lead structures that helped launch a new paradigm in antibody drug conjugate technology (currently under development by Centrose).

This glycosylation platform continues to present unique opportunities for improved formulation and targeted delivery.

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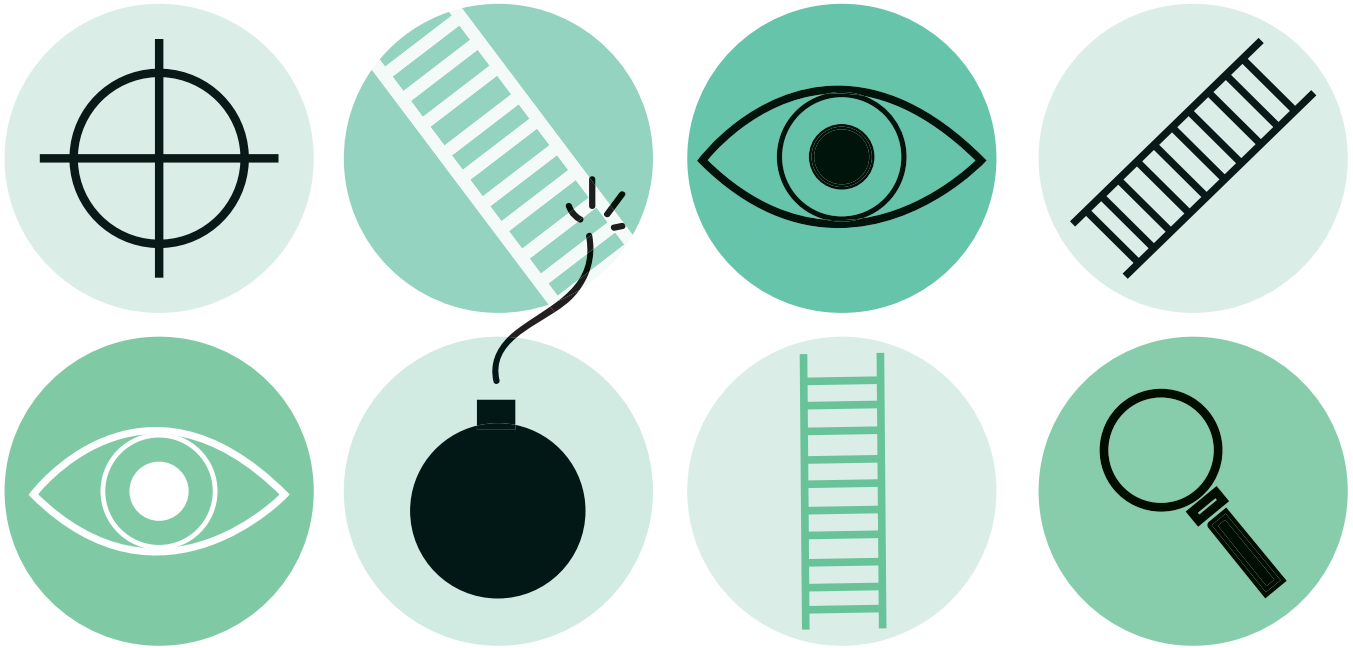
1. *SI Elshabawi et al., "Structure and specificity of a permissive bacterial C-prenyltransferase", Nature Chemical Biology 13, 366-368 (2017). PMID: 28166207*

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Catching Cargo Criminals

How does the FBI track down culprits of pharma thievery?

It's a heist story worthy of Hollywood. In 2010, a ladder was stashed in the rear parking lot of an Eli Lilly warehouse in Connecticut. Later that night, a tractor-trailer arrived and the ladder was carried to the building. Men climbed atop the warehouse and cut a hole in the roof. Using ropes, they lowered themselves into the facility and disabled the alarm system. And then stole \$60 million's worth of pharmaceuticals. Employees arrived later on to find the ladder, hole, discarded tools and the alarm system beeping, as if it needed a battery.

The thieves (Amed Villa, Amaury Villa, Yosmany Nunez – also known as “El Gato” – and Alexander Marquez) have all been caught; the latter three were sentenced in 2015 but Amed Villa wasn't

sentenced until December 2016.

In April 2017, the FBI revealed more details about the case, including what it takes to catch criminals (1) – here's our five-point summary:

- i. Believing that the thieves probably used a “follow car” in addition to the tractor-trailer, experts decided that the culprits most likely headed south, and would need to stop and rest after around 300 miles. Based on that information, the agents performed a logical investigation – locating points on maps, checking hotels, car rentals, airline reservations and cell phone tower analysis.
- ii. FBI agents and analysts trained in data analysis of electronic transactions focused on the follow car and where it came from.
- iii. Burglary tools were examined and the agents determined that the exact combination of gear left at the warehouse was purchased the night before at a big-box hardware store in Flushing Meadows, New York.
- iv. A plastic water bottle helped break the case – DNA from the bottle matched an individual in Florida with a history of cargo theft (Amed Villa).
- v. The FBI discovered that the culprits had stashed the drugs in self-storage units in Miami, which were then put under surveillance – though the agents believed it would be six months or more before the thieves tried to fence the drugs. In the meantime, the FBI tried to tie the thieves to other open cargo theft cases and succeeded. Amed Villa was tied to further incidents of cargo theft, including \$13.3 million in pharmaceuticals from the GlaxoSmithKline warehouse in Virginia in 2009.

Case(s) closed. *SS*

Reference

1. FBI, “Pharmaceutical Theft \$60 Million Heist Largest in Connecticut History”, (2017). Available at: <http://bit.ly/2nA7AGo>. Last accessed April 11, 2017.

Making a Difference to Drug Discovery

Differential mobility spectrometry aims to save R&D by making it cheaper, faster and more efficient

Early small molecule drug discovery uses cell permeability assays to determine the chemical structure and physicochemical properties of candidate molecules – but these in vitro studies are timely, expensive, and lack the resolution to discriminate between similar molecules. Enter differential mobility spectrometry (DMS)...

Though DMS is a technology known more for its ability to separate analytical

ions from chemical noise, researchers at the University of Waterloo were interested in probing how ions interact with solvent vapor. “We caught the attention of scientists at Pfizer, who were already collaborating with Sciex, and so we decided to merge (some of) our efforts,” says Scott Hopkins, a professor of chemistry at the University of Waterloo.

DMS uses oscillating electric fields to influence the motion and temperature of ions in the DMS cell. The researchers used this dynamic environment to drive rapid cycles of water condensation and evaporation (tens of thousands of times over the course of several milliseconds) and monitor the water vapor’s interaction with drug molecules. The interaction, the researchers found, correlates well to properties like solubility, pKa and cell permeability (1).

The technology could eliminate the

need for experienced technicians to perform a battery of tests to ascertain the properties of a potential drug. Instead, a single DMS analysis could obtain the same information – plus the ability to differentiate between structurally similar molecules – in a fraction of the time.

“The key thing from the drug discovery perspective is that we can make property measurements in seconds, with only picograms to nanograms of sample. Consequently, with a little more work, DMS could be used for cost-effective, high-throughput assays,” says Hopkins. *JS*

Reference

1. C Liu et al., “Assessing physicochemical properties of drug molecules via microsolvation measurements with differential mobility spectrometry,” *ACS Cent Sci*, 3 (2), 101-109 (2017). PMID: 28280776.

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Business-in-Brief

An opioid investigation, a new whistleblowing policy, and recalls galore... What's new for pharma in business?

Politics

- Members of the European Parliament have voted overwhelmingly to approve the European Council's Brexit guidelines, which say that "arrangements should be found" to facilitate the transfer of EU agencies located in London, such as the EMA, to alternative locations. Reportedly, 21 of the 27 EU member states have expressed an interest in hosting the EMA.
- Deaths due to overdosing on prescription opioids are on the rise in the US – and Senator Claire McCaskill is taking action by launching an investigation to find out how the country's leading opioid manufacturers are marketing their drugs. She has asked a number of companies for internal documents that discuss the risk of misuse, abuse, addiction and overdose, as well as documents outlining business and marketing plans, and quotas for sales representatives. In 2015, more than 15,000 people in the US died from overdoses relating to prescription opioids.

Regulation

- The EMA Board has adopted a new policy on whistleblowing – aiming to "create an environment where individuals from outside the Agency feel confident to raise their concerns on improprieties in their area of work". To this end, the EMA has created a dedicated



- email address, reporting@ema.europa.eu, where individuals can anonymously raise concerns. The agency has reportedly received "a total of 43 reports that relate, for example, to the manufacturing of medicines or the conduct of clinical trials," since 2013.
- President Trump's nominee to head the FDA, Scott Gottlieb, has pledged in his confirmation hearing to "remain faithful to the FDA's gold standard" for establishing the safety and effectiveness of new drugs, and that he would not approve drugs based only on safety. Gottlieb also said that price rises are enabled by the FDA's approval process and that this is a "solvable problem".

Manufacturing

- Mylan's recall of faulty EpiPens has spread to the US, after an international recall of around 81,000 EpiPens in Australia, New Zealand, Europe and Japan

took place in March. The recall] followed two reports of the life-saving allergy shot not working in emergencies – though in both situations patients were able to use an alternative EpiPen.

- GSK is voluntarily recalling nearly 600,000 Ventolin inhalers in the US because of a defect that may cause the inhalers to deliver fewer doses than indicated. The recall affects three inhaler lots that were manufactured in Zebulon, North Carolina.
- A strike over pension plans at a Pfizer plant in Ringaskiddy, Ireland, has ended after the company agreed to let 35 new staff access the firm's benefit pension scheme. The industrial action involved around 200 workers and lasted for more than eight weeks.

For links to press releases and source material, visit the online version of the article at: <http://tmm.txp.to/0417/business>

Bringing Time Back to Biology

Surface plasmon resonance has become a go-to tool for biotherapeutic characterization – and most approved antibody therapeutics have passed through a Biacore SPR system.

By Robert Karlsson

I became fascinated by analytical chemistry after working in a forensic laboratory as an undergraduate. Later, I joined Biacore where I worked on the development of surface plasmon resonance (SPR) – and I've been hooked on label-free biosensors ever since. What is SPR? It is a label-free, optical technique for analyzing molecular interactions. In our systems, we allow a target molecule on a functionalized metal film (the sensor chip) to interact with a ligand. By applying light to the sensor and by measuring the intensity of the reflected light as a function of the angle at which the light hits the sensor surface, an SPR response is obtained. This response is directly proportional to refractive index/mass changes at the sensor surface. By plotting response versus time in a sensorgram, a binding curve is obtained. From this, kinetics and affinity constants – parameters that are fundamental for the understanding of drug function – can be derived.

SPR is applicable throughout the whole drug development value chain. In the screening phase, hits can be identified by the shape of the binding curve. In later phases, drug candidates can be triaged on the basis of on- and off-rates; target occupancy is on-rate dependent, and drug residence time is off-rate dependent. On- and off-rates also shed light on target selectivity – for instance, compounds with identical kinase affinity can still be kinase-

selective because of off-rate differences.

The benefit of label-free analysis is that rather than making inferences from indirect measurements – as with end-point immunoassays, such as radioimmunoassays or ELISA – it directly analyzes binding events. The analysis also occurs in real time, allowing assessment of both associative and dissociative interactions. Additionally, SPR dispenses with wash steps typical of immunoassays, and therefore can characterize both low and high-affinity interactions. Finally, SPR can characterize both large and small molecule ligand-target interactions – a key advantage.

Bio characterization

SPR has become a popular method for characterizing biotherapeutics and biosimilars, partly because the technique can measure not only antibody-target binding, but also antibody interactions with Fcγ or FcRn receptors. Because these assays give important information on antibody molecular mechanisms of action and half-life, they are hugely important for measuring and monitoring critical quality attributes throughout the process – from cell line development to quality control – for all kinds of antibody products. Regulators emphasize comparison of biosimilars with approved reference products. Techniques such as liquid chromatography-mass spectrometry and capillary electrophoresis provide structural information, but SPR gives functional data on target and Fc-receptor binding, making it an ideal component of orthogonal functional analysis. SPR resonates favorably with regulators and is specifically mentioned in FDA guidelines.

Recently, we published an application note with Sartorius Stedim BioOutsource, who has incorporated a Biacore FcγRIIIa binding assay – predictive of antibody-dependent cellular cytotoxicity – into their orthogonal biosimilar functional characterization regime (1). This surrogate potency assay is applicable to development, manufacturing and quality control of antibody-based therapeutics.

The assay can assess multiple analytes in a single assay and return binding kinetics measurements that would be impossible for other methodologies. Sartorius Stedim BioOutsource use our technology to investigate a variety of biosimilar mAbs. To validate SPR, they correlate potency data from both SPR and a traditional cell-based method.

Over the last 30 years, I've helped develop Biacore systems from emerging instruments to drug discovery and development workhorses. We combine SPR, flow systems, advanced sensor surfaces and dedicated software in our intuitive, plug-and-play systems. Our newest system, Biacore 8K, has eight needles for simultaneous injections into eight flow cell pairs. Our most sensitive system, Biacore S200, is a one-needle system often used for small molecules. And Biacore T200 is our most versatile system, used for applications from R&D to quality control. Recently, we have introduced a sensorgram comparison tool for directly juxtaposing target and FcRn receptor binding in a single assay. This compares binding data, without determining rate and affinity constants, and complements dose response curves for potency analysis in biosimilar characterization.

One of the Biacore pioneers, Professor van Regenmortel, once said, "Kinetics brings time back into biology". It was an excellent point. Kinetic data allow prediction of interactions over time, which is crucial for our understanding of how molecules work and link to better understand biological processes. This is why I still love the technology and I hope to develop it further.

Robert Karlsson is staff scientist in the Purification and Analysis team at GE Healthcare Life Sciences.

Reference

1. <http://proteins.gelifesciences.com/discussion-forum/biacore-club/> (2017).



In My View

In this opinion section, experts from across the world share a single strongly held view or key idea.

Submissions are welcome. Articles should be short, focused, personal and passionate, and may deal with any aspect of pharmaceutical development or manufacture.

They can be up to 600 words in length and written in the first person.

*Contact the editor at:
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Going Granular

Microparticulates have many advantages and, though I agree there are also challenges, we must overcome these together to improve the patient experience.



By Nathan H. Dormer, Vice President of Research and Development at Orbis Biosciences, Lenexa, Kansas, USA.

The business model of pharmaceuticals has changed. The industry is no longer about blockbuster drug development, but about patient outcomes and product lifecycle management. This new era emphasizes efficiency in development and manufacturing – and, as a result, we’re all looking for cost-effective solutions that can expand a product’s applicability to a broader range of patients. Many new formulation approaches have been developed to create more patient-friendly medicines – from improving swallowability to reducing the number of doses that a patient must take.

One approach that I am very familiar with – and a current “buzzword” – is microparticulates. Either microspheres or microcapsules (offering a distinct “core” and “shell” layer), microparticulates are defined by a diameter in the sub-millimeter range. Think of them as micro-sized pills. Some of their advantages over larger, traditional capsules include greater flexibility in design, improved bioavailability and stability, and predictable gastric residence time, which means less inter- and intra-subject variability.

However, when considering the use of microparticulates in formulation, it’s easy to get bogged down by the countless manufacturing approach variations: spraying, spinning, congealing, extruding, coating, coating... and coating again. Multiple coating steps are applied to attain different release kinetics or behaviors from microparticulates. One layer may be used for drug stability, a second layer for pH-dependent release, and a third layer for taste-masking.

“I believe that microparticulate dosage forms can help enhance the user experience.”

The inclination to use microparticulates often arises when one or more incompatibilities exist between standard dosage formats (tablets, capsules, gel caps, caplets, and so on) and the terminal use profile. Such an incompatibility may exist in theory, formulation, manufacturing, packaging, stability, user experience, drug product performance, clinical efficacy, or long-term clinical outcomes. For example, microparticulates dosage forms can be used to help circumvent food effects because of the relatively small and dispersible size, or to help improve disintegration and dissolution speed. The size can also be a key formulation consideration to improve efficacy when delivering drugs to the small intestines.

But perhaps one of the most important

attributes of microparticulate dosage forms is their ability to address patient compliance by providing patient-friendly forms for those unable (or unwilling) to swallow a traditional oral solid. With patient and patient outcomes taking center stage, there is growing emphasis on catering to pediatric populations, as well as patients with dysphagia or organoleptic proclivities, or individuals suffering from serious mental illness. Traditionally, a liquid formulation would be used for these patient populations, but liquid forms are not always a good option, usually because of stability or taste considerations (the latter point being especially true for children). Microparticulate formats, however, can offer improved stability when delivered via a sachet format, or can be reconstituted immediately

prior to administration. They can also offer taste masking when the API is appropriately encapsulated.

Although microparticulates do have many advantages, there are some realities that need to be faced. Microparticulates are manufactured through a variety of different processes. Modified release is a popular use of microparticulates and generally relies on sequestration of the API via one or more physicochemical mechanisms, which require multiple manufacturing steps (just like traditional tablets). Formulating a powder, however, presents unique coating challenges because of the high surface area to volume ratio of particles, as well as the irregular overall diameters of particles within the powder bulk. The design and manufacturing required to ensure predictable performance and high quality

of the final product can be challenging, considering these constraints.

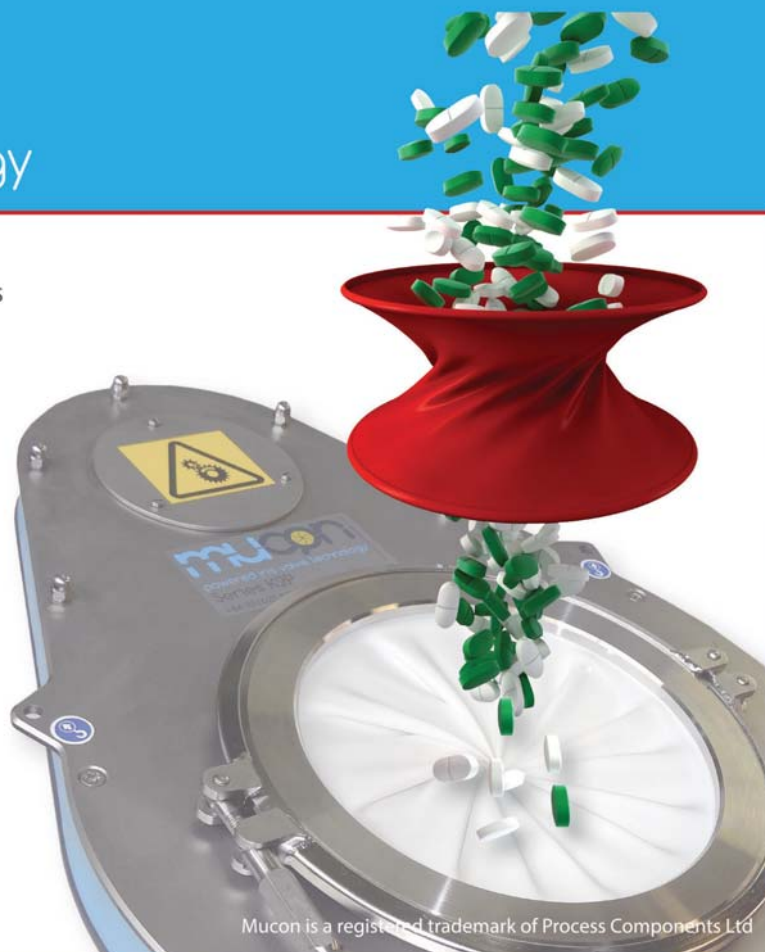
With any dosage form, clinical efficacy and long-term clinical outcomes all rely heavily on the drug being taken as designed and prescribed. Groundbreaking chemistry, cutting-edge stable formulations, manufacturing finesse, and ultra-cool product packaging are all meaningless if nobody ingests the product. Non-adherence is a significant problem – and it perpetuates patient suffering, increases clinician involvement, and results in unnecessary healthcare costs.

I believe that microparticulate dosage forms can help enhance the user experience – when made correctly. If your team decides that a particle format would be the perfect solution, then you should be very thoughtful about the

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design and execution, because so much relies on it. Does the microsphere need multiple layers of functionality? Is there a simpler way to achieve the result? Next-generation microparticulate technologies are offering simplified approaches that allow controlled release powders in a single-step process. Such controlled release powders can act

as a building block for a multitude of format options, which simplifies product life cycle management for pharmaceutical companies.

Microparticulates are emerging as a drug-delivery technology with significant benefits, not least their ability to produce patient-friendly products and superior patient outcomes. Right

now, too many underserved patient populations are crushing tablets or using extemporaneous formulations – and that can only lead to unnecessary patient risk. I believe that the industry has a duty to ensure that medicines are being used correctly – and that means that they need to be available in patient-friendly forms.

Clean Conundrum

Hygiene is essential in the pharma industry, but there are a surprising number of ways that cleaning – and its verification – can go wrong.



By Imad A. Haidar Ahmad, Principal Scientist, and Andrei Blasko, Senior Fellow, both in the Pharmaceutical and Analytical Development at Novartis Pharma, San Carlos Site, USA.

Many companies have been on the receiving end of FDA warning letters – and issues around cleaning arise time and time again. Failure to adequately clean equipment, failure to validate the cleaning verification method, failure to follow cleaning procedures... A quick reminder of the regulations: the cleanliness of non-dedicated equipment should be verified before subsequent release for use in the manufacture of intermediates and APIs, and at product change over to prevent cross-

contamination. The cleaning procedure must be validated according to FDA requirements (1), and companies must employ cleaning steps that are reproducible and effective.

“Cleaning verification has always been (and perhaps always will be) a challenge.”

When validating the analytical methods used for cleaning verification, the industry standard is to use stainless steel coupons with the same surface and finish as the manufacturing equipment. The sample of interest is spiked onto the stainless coupon at an amount determined by the maximum allowable carry-over (MACO) limit and recovered by swabbing or wiping the surface of the coupon. Spiked residues may include API, precursors of the drug substances, by-products, degradation products, cleaning agents,

and so on (2). The purpose of the cleaning verification is to prove that the residual contaminant is below its MACO limit.

Sounds straightforward, right? But in reality cleaning verification has always been (and perhaps always will be) a challenge, partly because of the analytical techniques used to determine trace analysis components. At such levels, several sources may contribute to variability, such as change in the sample composition, purity of reagents used, presence of microorganisms, matrix effect, adsorption, drug product degradation, or evaporation of the components of interest (3). Analysts are usually satisfied with a “cautious but good enough” approach rather than deep insight into understanding the sources of variability. In our recent study, recovery varied from excellent ($97\% \pm 1\%$) to failing ($50\% \pm 1\%$). The variability in results was observed at different spike levels (up to 23 percent difference between 50 and 150 percent cleaning limit, all other conditions being the same), at various ratios of API/excipient, among different analysts, and even for the same analyst on different days. We made a number of attempts to improve recovery by modifying the experimental conditions (swabbing technique, organic/aqueous ratio in diluent, the spiking solvent, and so on), but no change eliminated the observed variability.

Lack of understanding in variable recovery results is common, but we were not satisfied. We decided to investigate whether the coupon surface itself was a source of variability. The coupons were initially cleaned by rinsing and wiping the surface a few times with water and methanol to get rid of any residual deposit, but this did not help with improving recovery numbers. So the approach was modified to include fully immersing and sonicating the coupons in CIP100 and CIP200 solutions (a basic and acidic commercial cleaning agent, respectively) (4). By applying this cleaning procedure to the previously used coupons that failed the cleaning acceptance criteria, multiple analysts were able to obtain consistent recoveries from day-to-day for different APIs, and API/excipient ratios at

various spike levels. The approach was successfully applied for cleaning verification of small molecules (<1000 Da) as well as large biomolecules (up to 50,000 Da).

So what does this all mean? Our study showed that the lack of a well-defined procedure and cleaning agents for cleaning the coupon surface was the major contributor to low and variable recoveries. The low and variable recovery obtained from the uncleaned surface of the coupon may be caused by deposition of residual material on the surface, a change in the oxidation state of the surface of the coupon (enthalpic interactions with the sample are introduced), or both. Many companies no doubt experience variability in cleaning, which can be frustrating. It is worth taking the time

to better investigate variable recovery rates – it will likely result in cleaning and cleaning validation being far less stressful exercises.

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Why Are We Still Waiting for Biosimilars?

By applying “common sense,” President Trump has an opportunity to help get the industry moving and make a real mark on US healthcare by boosting biogeneric use.



By Robert Wessman, Chairman and CEO of Alvogen, USA.

Generic drugs saved around \$230 billion for the US healthcare system in 2016. For biosimilars, regulation has been in place for six years but no meaningful societal savings have been achieved, despite the great potential of these medicines. According to the RAND Corporation research institute, biosimilars alone could cut US spending on biologics by \$44 billion over the next decade (1).

Something has to give and, despite controversy, the Trump Administration might just prove to be the opening the industry has been waiting for. President Trump has suggested he wants to shake up pharma as part of his pledge to ‘Make America Great Again’. It’s difficult to tell how serious he is on the matter, but if he follows through on his claims then there is reason to believe that the route to market for biosimilars could be easier for manufacturers.

Trump would be wise to consider the latest projections from the Pharmaceutical Care Management Association (PCMA) before making any big decisions; PCMA’s report is not the first to suggest that

biosimilars could help finance Trump’s vision (2). The trade association claims its proposals would amount to \$100 billion in savings over a decade. Its recommendations include a shortened biologic exclusivity period – seven years instead of the current 12 (the number proposed by the Obama Administration when the biologics approval pathway was set up), which would bring biosimilar rivals to market much earlier.

PCMA has created a website to support its proposals, as well as taking to social media to spread the message. It also claims to have met with “key officials all over Washington” to talk drug prices. A call for greater biosimilar use is just one of the proposals being pushed by PCMA – the association is also calling for an end to requirements for insurance plans to pay for all drugs in certain classes under Medicare, regardless of price.

However, my concern is that the biosimilars sector will continue to be held back by a lack of interchangeability guidance and ongoing litigation. Though the Biologics Price Competition and Innovation Act (BPCIA), which created a regulatory pathway for biosimilar products in the US, looks to be safe from Trump’s dismantling of the Affordable Care Act, biosimilars are not likely to be high on the administration’s agenda. The Generic Pharmaceutical Association (GPhA) is doing its best to present biosimilars as a “ready-made solution” to high drug costs, but new measures are desperately needed to aid their entry into the market. As a starting point, here are three measures that could go a long way towards boosting biosimilars use in the US.

Firstly, implementation of interchangeability. In January, the FDA released its long-awaited draft guidelines detailing the agency’s expectations for demonstrating biosimilar interchangeability. The guidance recommends that sponsors conduct one or more switching studies to demonstrate safety and efficacy in patients alternating between the two products. However, the FDA has stated

that requirements will vary based on the nature of the proposed interchangeable product and may include an evaluation of data and information generated to support a demonstration of a biological product’s biosimilarity. In short, we’re in for a long wait before an interchangeable biosimilar comes to market... As I see it, substitution was the key for small molecules and will be for biosimilars, too.

Secondly, launch at approval. Biosimilar approvals are progressing, but significant hurdles remain, even when a biosimilar is proven to be highly similar to its reference product in the shape of stays and patent-linkage. Currently, biosimilar makers must wait six months after winning federal approval to begin selling them – although this may change. The US Supreme Court has agreed to hear a dispute over whether companies must wait that long. Is it right that brand-name manufacturers are given an extra six months of exclusivity on top of the 12 years already provided for under the law, driving up healthcare costs?

Thirdly, pro-generic penetration mechanisms. We need to implement mechanisms that further support generic penetration and allow free competition – it worked for small molecule generics and it will for biogenerics, too. I don’t need to highlight how American brand and specialty drug prices are among the highest anywhere (despite the fact the US pharmaceutical market is the largest in the world). Pro-generic penetration mechanisms would appear to be common sense to anybody looking in.

On that note, Trump has defended some of his policies as “common sense”; I wonder if he intends to apply that to biosimilars, too?

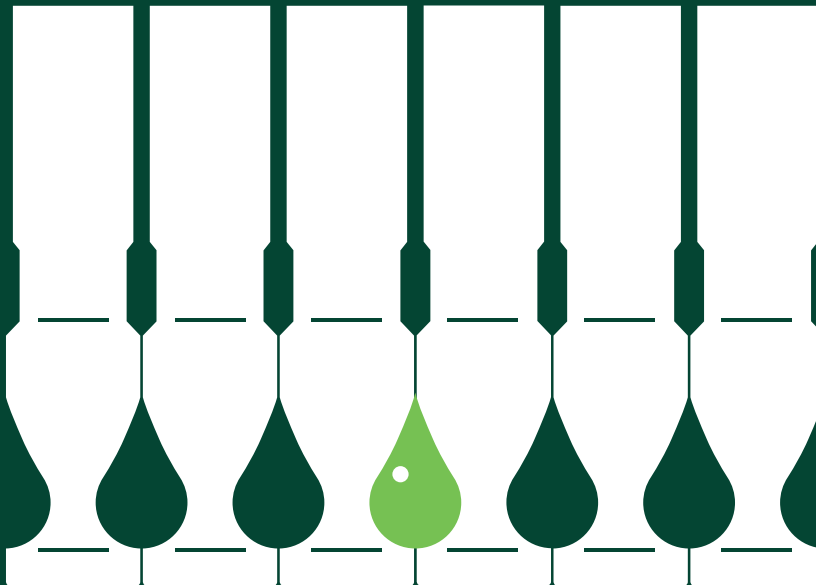
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Making the Media of the Future – Today

Biopharma manufacturing continues to advance and the demand for new innovations, such as more efficient cell culture media, is constant.

In previous articles in this series, we heard about intensified perfusion systems from Delia Lyons (1), and about high-density cryopreservation from Jochen Sieck (2). Now, Lyons is joined by Jeremiah Riesberg (Senior Scientist, Perfusion Medium Development, Millipore Sigma, USA) to discuss a novel culture medium. The product, currently in beta testing with chosen clients, has been designed for the most challenging of applications – intensified perfusion at steady state – but is also applicable to a range of other processes.

Why is continued innovation in cell culture media so crucial?

Delia Lyons: Many advances have been made in cell culture media but as the biopharma industry evolves, its needs change – this means we need to continue pushing the boundaries of innovation. Today, clients are demanding faster growing cell lines, higher volumetric productivity, improved product quality, and advanced processes for innovative molecules. In turn, these needs drive demand for improved or entirely novel cell culture media.

We make great efforts to be proactive. Our aim is to anticipate the needs of the market so that we have products ready when the market requires them. The ability to understand the industry before the industry understands itself is essential if you want to be a market

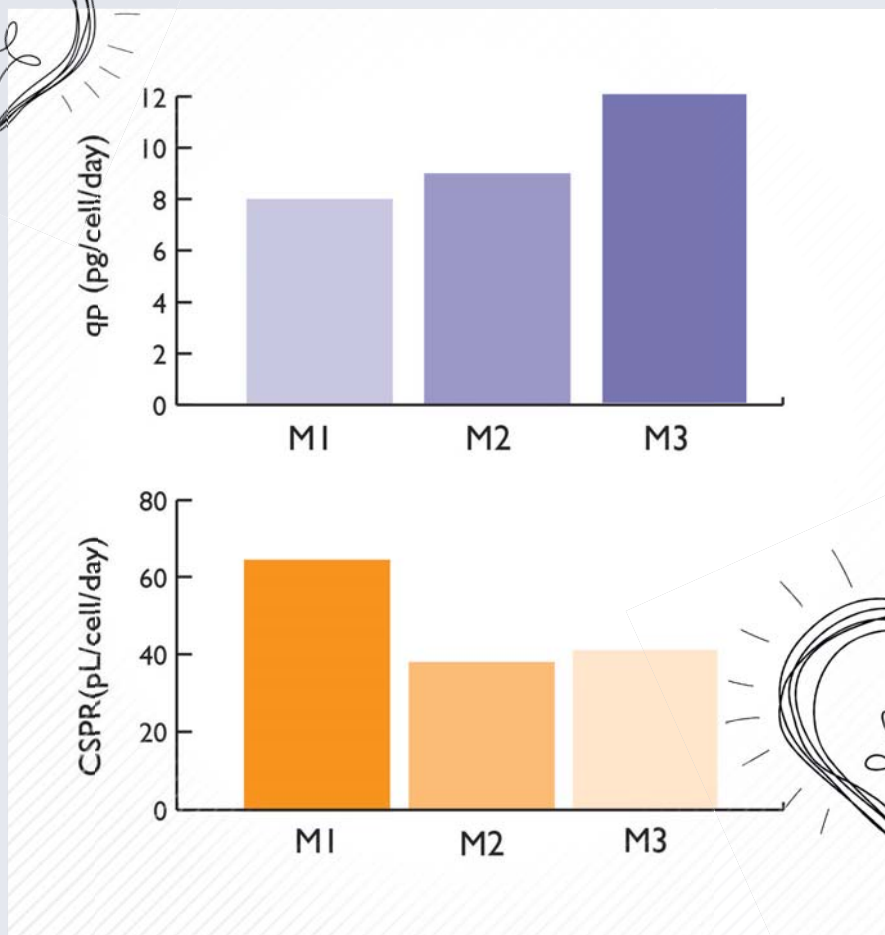


Figure 1: Final medium (M3) could maintain a target cell density with a 40pL/cell/d with a 50 percent increase in specific productivity compared to prototype M1.

leader. However, getting to this position requires a high degree of customer intimacy and close, trust-based client relationships; customers need to feel comfortable sharing future plans and needs, and to know that we listen and consider their input when planning our product development roadmap.

How do available media differ from each other?

DL: In chemical terms, most commercially available media have similar critical components – they all need a particular set of nutrients to meet the basic metabolic demands of cells. One very important distinction between different media is the

quality and reliability of the raw materials used in their production. For example, a given nutrient may be available in different chemical forms – and each form may affect cells differently or have lot to lot variability, so it is extremely important to choose the right one. Our raw material characterization team is crucial to ensuring that our products meet the relevant quality standards.

Jeremiah Riesberg: There are many different cell lines, products, and processes in the industry, so the market needs a range of media that can support these different systems. Different biomanufacturing processes, such as fed-batch and perfusion, have unique characteristics, so

well balanced medium is desirable to get the best performance from the specific process. It is critical to understand the intended use of the medium, and what needs it must meet.

What is the intended application of your new medium?

JR: Our new catalog medium, EX-CELL® Advanced™ HD Perfusion Medium, has been specifically designed for intensified processes, and tested with steady state perfusion, which is considered very challenging. Maintaining consistent product quality and yield at very high cell densities can be difficult, especially over time periods of 30 or more days. I believe that many companies would welcome a medium that addresses steady state perfusion challenges, and it will be available to them this summer. Furthermore, we expected that a medium that performs well for this most demanding application – maintaining constant productivity over long periods at very low cell specific perfusion rates – would also be applicable to other perfusion-based intensified processes.

How does the new medium compare with the ‘industry standard’?

DL: There is no industry standard! This is the first commercially available medium specifically designed for perfusion processes, so there is no true benchmark yet. We developed it from scratch, using our standard media development tools. We used multivariate statistical analysis to really understand the effect of individual components of the medium. Some components that are beneficial in fed-batch turn out to be excessive in continuous perfusion – more isn’t always better – so to understand which components needed reduction, we had to interrogate individual components of the medium. In other words, we wanted to thoroughly understand the chemistry of the medium and how it

affects cells. We tested many different improvements on existing basal media, but the novel medium we developed by optimizing individual components was superior to this. Our product is not just a modified fed-batch medium – and I believe this is a key differentiator.

JR: When we compared our de novo medium to others developed using the traditional approach (adding feed to a basal fed-batch medium), we found the new medium could simultaneously increase the yield by 50 percent while reducing the cell-specific perfusion rate (CSPR) to 40 picolitres per cell per day (pL/cell/day) (Figure 1). With some cell lines, we were even able to maintain a steady state with a CSPR of 20 pL/cell/day.

What difficulties did you face during development?

DL: It is always difficult to understand and predict current and future market requirements. When you develop an entirely new product, there’s no history to use as a guide – you have to work things out as you go along. We had a number of practical issues to contend with; for example, new media development requires sufficient throughput to enable a reasonable number of conditions to be tested at a small enough scale to remain economically feasible – otherwise you cannot quickly learn and progress.

JR: Exactly. We had to modify our workflow because there was no established small-scale perfusion model or cell separation device with small enough dimensions available. Developing a scaled-down system enabled us to apply the statistical tools that we use to assess the effects of individual components of medium and optimize them specifically for steady state perfusion applications. Developing a small-scale perfusion system in parallel with the development of the medium was a significant challenge.

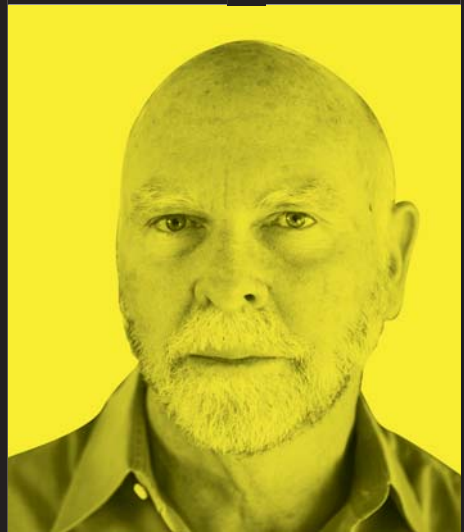
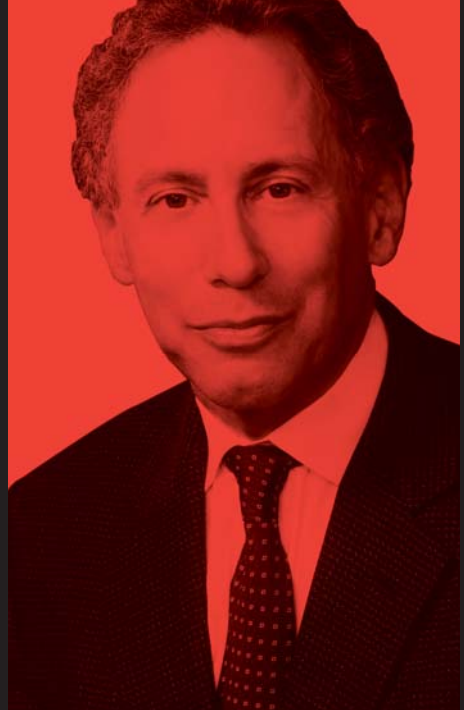
As a team, we all worked very hard on this – and we are very proud of what we have accomplished. I think it is particularly important to note the breadth of applicability. We developed this medium with seven different CHO cell lines producing different types of proteins. From those, four industrially relevant cell lines have been evaluated in steady-state perfusion bioreactors. One of the advantages of Merck KGaA is our in-house cell line development team, who have been invaluable in providing us with the resources to show that the new medium is compatible with a wide range of important cell lines.

How else is Merck KGaA innovating to meet evolving industry needs?

DL: We appreciate that intensified perfusion applications – steady state or not – are new to the industry, and many clients will benefit from support when they first implement these novel processes. Of course, some companies have been using traditional perfusion processes for labile proteins for years and have a great deal of experience, but many others are relative newcomers. One of our valuable resources is our global team of experts with first-hand experience helping clients set up, optimize, and customize perfusion processes. Today, it is not enough to just develop innovative products – companies should always be looking at how they can add value. We are proud to be part of the teams that offer support and training to customers, helping advance industrial practices and trends.

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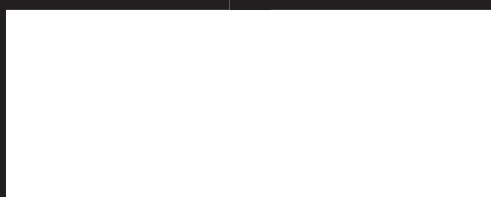
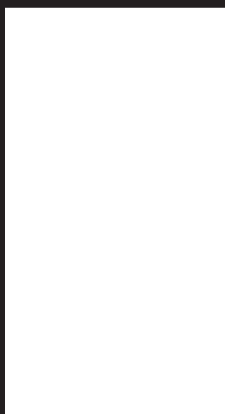
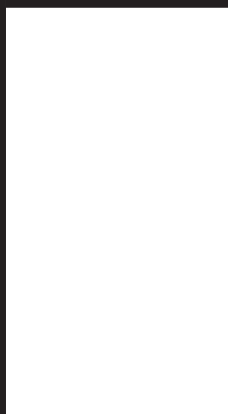




the
Medicine Maker

Power List

2017



With two somewhat provocative Power Lists behind us, we once again forge ahead with our mission to celebrate the fantastic individuals involved in bettering the pharma industry, and in bringing life-changing medicines to market. Welcome to the 2017 Power List, where we delve into the passions, pivotal moments and predictions of the Top 100 most influential people in the industry divided into four categories – each made up of 25 names and led with a Top 10: Masters of the Bench (page 24), Industry Influencers (page 31), Business Captains (page 37), and Champions of Change (page 43). The overall Top 10 can be found on page 48.



MASTERS OF THE BENCH



10. CHAS BOUNTRA

PROFESSOR OF TRANSLATIONAL MEDICINE, DIRECTOR OF STRUCTURAL GENOMICS CONSORTIUM, AND DIRECTOR OF OXFORD MARTIN SCHOOL, UNIVERSITY OF OXFORD

“I am excited by our goal of discovering and developing new medicines for patients, as well as the opportunity to work with amazing people every day. Pharma is building increasingly porous relations with academia. I am sure this will foster a broader appreciation of the difficulties we face, change the reputation of the industry for the better, and accelerate the development of novel medicines for society. I also hope these new medicines will be more affordable.”

7. JOSEPH SCHLESSINGER

WILLIAM H. PRUSOFF PROFESSOR AND CHAIRMAN OF THE PHARMACOLOGY DEPARTMENT, YALE SCHOOL OF MEDICINE; DIRECTOR, YALE CANCER BIOLOGY INSTITUTE

Joseph is an internationally recognized expert in the field of cell signalling and in personalized treatment for cancer. He is known for the discovery of receptor dimerization, which explains how receptor tyrosine kinases are activated when a molecule binds at the cell surface. He was recently named to the 2016 Class of Fellows of the American Association of Cancer Research Academy.



9. SUZANNE FARID

PROFESSOR OF BIOPROCESS SYSTEMS ENGINEERING, UNIVERSITY COLLEGE LONDON

Suzanne’s research centers on computer-based, decision-support tools—particularly those that help with the design of cost-effective bioprocesses, such as tools that can analyze economic drivers and trade-offs in antibody production, or tools that help with capacity planning and portfolio management.



6. SHINYA YAMANAKA

DIRECTOR AND PROFESSOR, CENTER FOR IPS CELL RESEARCH AND APPLICATION (CIRA), KYOTO UNIVERSITY

Shinya has made The Power List for the third year running for his pioneering work on stem cells. After reprogramming adult mouse (2006) and human (2007) somatic cells into what are now called induced



8. CHRISTOPHER J.H. PORTER

PROFESSOR, MONASH INSTITUTE OF PHARMACEUTICAL SCIENCES, MONASH UNIVERSITY

“Scientifically, the realization that the interaction between drug formulations and digestive enzymes in the gut can dictate subsequent patterns of absorption, and that directing drugs to the lymphatic system can have both pharmacodynamic and pharmacokinetic consequences, has driven much of the activity in my lab over the last 15 years. Beyond that, a key determinant of the impact of my work has been my good fortune to work with a fantastic group of academic and industrial collaborators.

“I realize that this is a utopian and perhaps unrealistic aim, but I’d like to see a greater emphasis on addressing unmet medical need in a manner that is not driven by market size. If the lack of new solutions to combat antimicrobial resistance has taught us anything, it is perhaps that leaving the direction of drug discovery to what is most profitable, while understandable within current commercial models, is not always in our best interests.”



pluripotent stem cells, he was awarded a Nobel Prize in 2012.

5. NICHOLAS A. PEPPAS

PROFESSOR AND DIRECTOR OF THE INSTITUTE FOR BIOMATERIALS, DRUG DELIVERY AND REGENERATIVE MEDICINE, THE UNIVERSITY OF TEXAS AT AUSTIN

“What is my passion? First, developing new therapeutic systems based on proteins to treat patients suffering from diabetes, autoimmune diseases and gastrointestinal

diseases. Second, developing new patented technologies for the treatment of disease through the identification of potential biomarkers. As for pivotal career moments, the announcement of the first heart transplantation by Christiaan Barnard at the Groote Sure in Cape Town, South Africa, on December 5, 1967, was an important moment for me – since it affected my career choice. And of course, the development of an “intelligent” polymer

(hydrogel) carrier based on a response to external physiological conditions, in my lab in December 1976.”



4. DAVID BALTIMORE

PRESIDENT EMERITUS AND ROBERT ANDREWS MILLIKAN PROFESSOR OF BIOLOGY, CALIFORNIA INSTITUTE OF TECHNOLOGY

David Baltimore’s love for experimental science was born after spending a summer at the Jackson Laboratory in 1955. And since then his achievements have been numerous, including a Nobel Prize in 1970 for the discovery of reverse transcriptase, which implied that cancer could be caused by genetic means – a wide-open question at the time.



3. PHIL S BARAN

DARLENE SHILEY PROFESSOR, THE SCRIPPS RESEARCH INSTITUTE

Phil has racked up a number of impressive natural product syntheses, and won dozens of chemistry’s highest accolades. His passion is “fundamental chemistry with rapid translational potential” and a pivotal moment was when he realized “one can’t trust in public funding alone to finance a lab.” Phil would like to see more partnerships with academic labs, and the appointment of chemists to leadership positions.

1. ROBERT SAMUEL LANGER

INSTITUTE PROFESSOR, MASSACHUSETTS INSTITUTE OF TECHNOLOGY

Considered one of the most prolific inventors in medicine, Robert Langer has over 1100 issued and pending patents. He previously served on the FDA’s Science Board and has been elected to the Institute of Medicine of the National Academy of Sciences, the National Academy of Engineering, and the National Academy of Inventors. Robert says he is passionate about working with fantastic students and he would like to see more basic long range research.

2. CARL JUNE

RICHARD W. VAGUE PROFESSOR IN IMMUNOTHERAPY, UNIVERSITY OF PENNSYLVANIA

Carl is also Director of the Center for Cellular Immunotherapies at the Perelman School of Medicine, Medicine Director of Translational Research Programs and Director of the Parker Institute for Cancer Immunotherapy, both at the University of Pennsylvania. He is also co-founder and chief scientific advisor of Tmunity



Therapeutics. Carl and his fellow “CRISPR Pioneers” were named as runners up in Time’s 2016 Person of the Year.



DAVID BENTLEY

VICE PRESIDENT AND CHIEF SCIENTIST, ILLUMINA

David was a founding member of the Sanger Centre (now the Wellcome Trust Sanger Institute in the UK) and led the Centre in their contributions to the Human Genome Project. He and his team have been involved in the 100,000 Genomes Project in partnership with Genomics England and the UK National Health Service.



JONATHAN BONES

PRINCIPAL INVESTIGATOR, NIBRT CHARACTERIZATION AND COMPARABILITY LABORATORY, NIBRT - THE NATIONAL INSTITUTE FOR BIOPROCESSING RESEARCH AND TRAINING

“My research is all about delivering solutions to enable people to better understand their processes and products, and to empower them to deliver better medicines to benefit patients that need them. There are a lot of exciting things going on currently. My group is actively working on extractable and leachable analysis of single use bioprocessing solutions and the data is fascinating. We hope that, once published, it will really help the industry in their choices to adopt and implement new technologies with confidence.”

JONAS BOSTROM

PRINCIPAL SCIENTIST, ASTRAZENECA

“I love combining new technology and science in innovative ways, with the grand purpose of treating diseases. I particularly like doing cool (and useful) stuff with computers – usually related to chemistry and/or life science. We’ve covered a very diverse range of research areas, from analysis of past and present synthetic methods, deriving matched-pair SAR methods, developing disruptive virtual screening capabilities, to the development of new virtual reality platforms for drug designers.”



MEINDERT DANHOF

PROFESSOR OF PHARMACOLOGY, LEIDEN UNIVERSITY

Meindert Danhof’s research focuses on novel concepts of systems pharmacology,

interfacing theories from systems biology with quantitative pharmacology. He is former scientific director of the Leiden Academic Center for Drug Research, and a Past President of the European Federation of Pharmaceutical Sciences.

KENNETH GETZ

ASSOCIATE PROFESSOR AND DIRECTOR, CENTER FOR THE STUDY OF DRUG DEVELOPMENT, TUFTS UNIVERSITY SCHOOL OF MEDICINE; FOUNDER AND BOARD CHAIR, CISCRP

“I am keen to solve the mysteries associated with poor, inefficient drug development

processes and to optimize performance to deliver meaningful and relevant new therapies to patients. I would like to see the drug development industry commit to returning clinical trial results summaries directly to all study volunteers as a standard practice, as a way to formally express appreciation for their gift of participation. Today, 90 percent of study volunteers never learn about the results of their own studies.”





HELEN H. HOBBS

PROFESSOR, INTERNAL MEDICINE AND MOLECULAR GENETICS, AND DIRECTOR, EUGENE MCDERMOTT CENTER FOR HUMAN GROWTH AND DEVELOPMENT, UNIVERSITY OF TEXAS SOUTHWESTERN MEDICAL CENTER

Helen was awarded the Breakthrough Life Sciences Prize in 2015 for her work on identifying key genes involved in lipid metabolism and fatty liver disease. In 2016, she was awarded the Passano Award and the Gill Award for her work on cardiovascular care.



CLAUS-MICHAEL LEHR

HEAD, DEPARTMENT OF DRUG DELIVERY, HELMHOLTZ-INSTITUTE FOR PHARMACEUTICAL RESEARCH SAARLAND, HELMHOLTZ CENTER FOR INFECTION RESEARCH (HZI), SAARLAND UNIVERSITY

“I am passionate about the convergence of scientific challenges required to safely deliver drugs across biological barriers. Drug delivery is like a triathlon; you must be good in more than one discipline – biology, chemistry, physics, medicine and engineering – to reach your goal. When we started our new Helmholtz Institute a few years back, I realized that there are many unexplored needs and opportunities for advanced drug delivery research in the context of infectious diseases.”



AARON KESSELHEIM

ASSOCIATE PROFESSOR, MEDICINE, HARVARD MEDICAL SCHOOL, AND DIRECTOR, PROGRAM ON REGULATION, THERAPEUTICS, AND LAW (PORTAL), DIVISION OF PHARMACOEPIDEMIOLOGY AND PHARMACOECONOMICS, BRIGHAM AND WOMEN'S HOSPITAL

“I use empirical methods to study drug development, approval and use, with the goal of informing evidence-based policymaking in the pharmaceutical market. A key moment for me was the creation of my PORTAL program in 2012 to help build a center of activity and a cadre of researchers interested in pharmaceutical law and health services research.”



PAULINE RUDD

PRINCIPAL INVESTIGATOR, NIBRT – THE NATIONAL INSTITUTE FOR BIOPROCESSING RESEARCH AND TRAINING

Pauline’s passion for glycans started early – extracting sugars from natural products in her kitchen as a teenager. Now at NIBRT, her research group focusses on developing advanced glycoanalytical technologies to analyze glycosylation in biotherapeutics and systems biology. In 2010, she was awarded the James Gregory Medal and an Agilent Thought Leader award.



DOLORES SCHENDEL

CHIEF EXECUTIVE OFFICER AND CHIEF SCIENTIFIC OFFICER, MEDIGENE AG

Dolores has been a member of the German Research Foundation, German Cancer Aid and the European Research Council. She joined Medigene in 2014, when the company acquired Trianta Immunotherapy. She is passionate about bringing individualized immunotherapies from bench to bedside by moving herself and her team from in-depth research to the forefront of the biotech industry.

PETER SEEBERGER

DIRECTOR, MAX-PLANCK-INSTITUTE OF COLLOIDS AND INTERFACES; PROFESSOR, FREE UNIVERSITY OF BERLIN

“Early on, I received a Fulbright scholarship to perform graduate work at the University of Colorado with Marv Caruthers, the inventor of gene synthesis machines and a co-founder of Amgen. It opened new perspectives and eventually led to my work on automated glycan assembly, which in turn resulted in novel vaccines and diagnostics. My work



today focuses on creating vaccines to protect everybody from deadly infectious diseases, and new methods to produce affordable drugs.”

ANDREAS SEIDEL-MORGENSTERN

DIRECTOR, DEPARTMENT OF PHYSICAL AND CHEMICAL FOUNDATION OF PROCESS ENGINEERING, MAX PLANCK INSTITUTE FOR DYNAMICS OF COMPLEX TECHNICAL SYSTEMS

Andreas’ research focuses on developing concepts to better link the various steps involved in drug production. He has published close to 400 research papers and has over 70 patents to his name. “Our current work is devoted to developing concepts for rationally designing combinations of separation processes so that we can efficiently isolate target molecules from complex mixtures,” says Andreas.



MICHAEL SOFIA

CHIEF SCIENTIFIC OFFICER, ARBUTUS BIOPHARMA

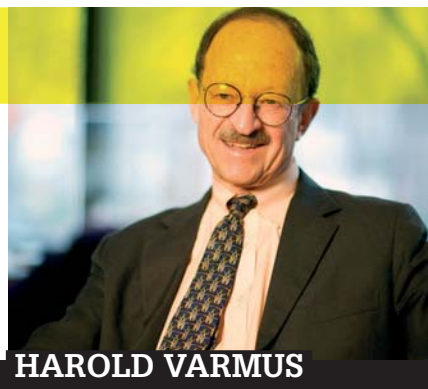
“There are few fields where one can come into work every day and know you have the chance to positively change how we treat human disease. My passion for this field lies in tackling the daunting problems in biomedical research and making an important contribution to finding solutions. I decided to leave the comforts of a big pharma position and join a small company developing antivirals. This company had no products, little money and a fledgling research group with no defined programs. It was a huge risk that many advised me not to take, but I saw great potential in a nascent program in hepatitis C. It was because of that pivotal decision that sofosbuvir was ultimately born.”



JOHN TALLEY

CHIEF SCIENTIFIC OFFICER, EUCLISES PHARMACEUTICALS

“My passion is unraveling the role prostaglandins play in immune surveillance within the tumor microenvironment. I would also like to see a return to the highly-regarded reputation of the pharmaceutical industry. This might be accomplished by promotion of the many tangible benefits to individuals that are a consequence of the industry’s research and development initiatives.”



HAROLD VARMUS

LEWIS THOMAS UNIVERSITY PROFESSOR, WEILL CORNELL MEDICAL COLLEGE

Harold Varmus has previously been the director of the National Institute of Health and the National Cancer institute. In 1989, he was the co-winner of a Nobel Prize (alongside Michael Bishop) for the discovery of the cellular origin of retroviral oncogenes – how malignant tumors are formed from normal cells.



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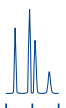
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HUMANITY IN
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**Wanted:
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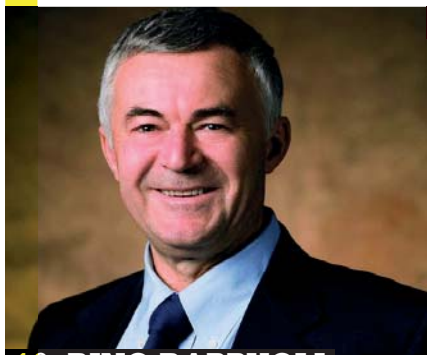
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www.humanityinscienceaward.com*

Nominations close on May 1st 2017

INDUSTRY INFLUENCERS



10. RINO RAPPUOLI

CHIEF SCIENTIST AND HEAD OF EXTERNAL R&D, GLAXOSMITHKLINE VACCINES

Rino is the author of over 600 research papers and has introduced a number of novel scientific concepts that have had a widespread impact on the vaccines industry. He is also the project coordinator of ADITEC (advanced immunization technologies).



9. SIR ANDREW WITTY

FORMER CEO OF GLAXOSMITHKLINE; CHANCELLOR, UNIVERSITY OF NOTTINGHAM

Sir Andrew graduated from the University of Nottingham in 1985, with a BA in Economics, and joined GlaxoSmithKline the same year. He was appointed CEO in 2008 – a position he held until April 1, 2017. He has served as Chancellor of the University of Nottingham since 2013.



8. MAIK JORNITZ

CHIEF EXECUTIVE OFFICER, G-CON MANUFACTURING INC

“A pivotal moment in my career was meeting my mentor, Theodore H. Meltzer, in 1991 at a filtration conference in London. He opened my scientific mind and encouraged me to share my experience through writing, presenting and getting engaged in expert groups. He also introduced me to the PDA – and my 20 year membership widened my knowledge horizon tremendously.”



7. JOHN E. BOURNAS

PRESIDENT AND CHIEF EXECUTIVE OFFICER, INTERNATIONAL SOCIETY FOR PHARMACEUTICAL ENGINEERING (ISPE)

John has previously worked as a Senior Director of International Affairs at the American College of Cardiology and at Cardinal Health. John has an MA in Political Science and was involved in foreign affairs earlier in his career.



6. GIL ROTH

PRESIDENT, PHARMA & BIOPHARMA OUTSOURCING ASSOCIATION

Gil says he is trying to change the outsourcing landscape – founding PBOA in 2014 to give CDMOs a unified voice within the industry. He has also been involved in discussing the reauthorization of GDUFA with the FDA. Gil has a literature degree and was Editor of Contract Pharma Magazine from 1999 to 2014.



5. GUIDO RASI

EXECUTIVE DIRECTOR, EUROPEAN MEDICINES AGENCY

Guido is serving his second term as Executive Director of the EMA, having served as the EMA's Principal Adviser in Charge of Strategy between terms. He has previously worked as a physician and has published more than 100 scientific papers.



4. MONCEF SLAOU

FORMER CHAIRMAN OF VACCINES,
GLAXOSMITHKLINE

Moncef was professor of immunology at the University of Mons before his move into industry. He has published more than 100 scientific papers and is a member of the International AIDS Vaccine Initiative Board of Directors. Moncef was at the helm of GSK's global vaccines business from 2015 to 2017.



3. ANDREW D. SKIBO

HEAD OF GLOBAL BIOLOGICS
OPERATIONS & GLOBAL
ENGINEERING, ASTRAZENECA/
MEDIMMUNE

"I am passionate about ensuring that we (both AstraZeneca/MedImmune specifically, and our biopharmaceutical industry as a whole) have a long range biologics supply capability," says Andrew. He would like to see the pharma industry work closer with regulatory agencies to find ways to dramatically reduce the cost of developing new drugs – specifically the cost of clinical trials – without sacrificing product safety or affecting patient risks.



2. MARTIN VAN TRIESTE

CHAIRMAN OF THE BOARD,
PARENTERAL DRUG
ASSOCIATION

"I started my career in R&D as a formulation pharmacist. I envisioned spending most of my career at the bench or in R&D management, but early in my career I ended up transitioning to product complaints. The new role exposed me to all parts of the company, not just R&D and quickly led to my advancement in various operations roles culminating as the Chief Quality Office of Amgen.

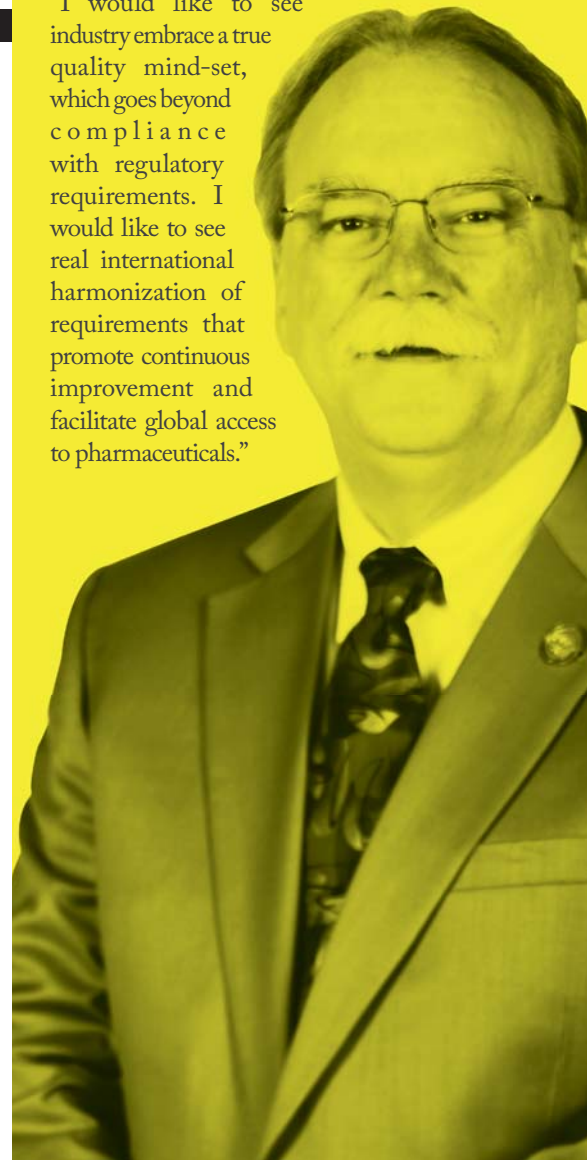
"I believe the industry will begin a major transformation over the next five years. We have entered into what many have called the biotechnology century. By cracking the human genetic code, we have unleashed a movement that will advance medicine faster than ever before in human history. With this vast knowledge, new drugs and therapies will take less time to develop, test in clinical studies and gain regulatory approval. These new medications and therapies will require an entirely different manufacturing and distribution system. In just 10 years, I believe that virtually none of the current manufacturing and distribution systems will exist."

1. RICHARD M. JOHNSON

PRESIDENT AND CHIEF
EXECUTIVE OFFICER,
PARENTERAL DRUG
ASSOCIATION

"I became active in PDA 25 years ago, and this has given me the opportunity to help lead the way in promoting science-based solutions for challenges the industry faces, and to work with health authorities to improve the understanding and implementation of best practices. Serving patients is and should always be the focus of our efforts. At PDA, I am committed to working to advance our knowledge, promote best practices, and drive quality through collaboration between all stakeholders: manufacturers, suppliers and health authorities.

"I would like to see industry embrace a true quality mind-set, which goes beyond compliance with regulatory requirements. I would like to see real international harmonization of requirements that promote continuous improvement and facilitate global access to pharmaceuticals."





JAMES AGALLOCO

PRESIDENT, AGALLOCO
& ASSOCIATES

James' passion is combining scientific principles and engineering insights to innovate in pharmaceutical manufacturing. "Other industries have leveraged technology to a far greater extent than have pharmaceuticals, and have made great strides in both quality and efficiency. The biopharma industry can dramatically improve if it emulates what has been accomplished elsewhere."



CARSTEN BROCKMEYER

CHIEF EXECUTIVE OFFICER,
FORMYCON AG

Carsten is passionate about creating a healthier world by widening access to state-of-the-art therapies. "A pivotal moment for me was designing the strategy and leading the cross-functional team for the development and launch of the world's first complex biosimilar, Binocrit (epoetin alfa), in 2007." He adds that he would like to see a more collaborative approach to drug development in the future.



JOHN AUNINS

EXECUTIVE VICE PRESIDENT OF
BIOPROCESS & MANUFACTURING
AND CHIEF TECHNOLOGY OFFICER,
SERES THERAPEUTICS

Before joining Seres, John led process and product development teams at Merck Research Laboratories for a number of products including Vaqta, Varvivax and Gardasil. He is a Fellow of the American Institute for Medical and Biological Engineering, as well as an adjunct Full Professor at the Instituto de Tecnologia Química e Biológica in Portugal.



STEPHEN T. COLGAN

SENIOR DIRECTOR, PFIZER
WORLDWIDE RESEARCH AND
DEVELOPMENT

"I am very proud of my work with the Analytical and Pharmaceutical Quality Executive Team (APQ ET) of the AAPS. The delivery of safe and efficacious medicines in a global environment is greatly facilitated when companies work with each other, and with regulatory authorities. As part of the APQ ET, I was able to identify individuals with similar interests and passions and then work with them on



JIM BREEN

VICE PRESIDENT, LEAD BIOLOGIC
EXPANSION, JOHNSON & JOHNSON

"Accepting a position that required my relocation to China in the mid-1990s changed my career and provided an education not learned in any course or program. Experiencing a new culture and appreciating new perspectives was a tremendous experience that continues to influence my decisions and career." Jim predicts that during the next five years, pharma will become more globally connected, and that the speed and availability of data will drive faster decisions in pharma research, development and manufacturing."



common goals. With the help of many colleagues in the community of stability scientists, global regulatory guidance and practices have (and continue) to evolve, which is great news for our patients."



FIONA GREER

GLOBAL DIRECTOR, BIOPHARMA SERVICES DEVELOPMENT, SGS

“A pivotal moment came early in my career, when I joined a small start-up company exploiting the then novel technique of fast ion bombardment mass spectrometry to sequence proteins and carbohydrates. In addition to developing the methods on new types of instruments, I was working to apply them to structural problems with colleagues in the emerging biotech industry. It was an exciting time to be developing new analytical technologies!”

JOHN LAMBERT

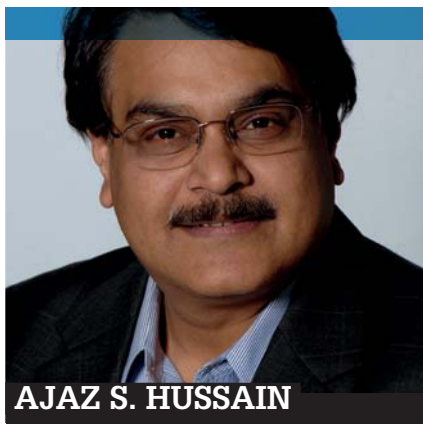
EXECUTIVE VICE PRESIDENT EMERITUS & DISTINGUISHED RESEARCH FELLOW, IMMUNOGEN

John was the second scientist to be hired at ImmunoGen in the 1980s and spent most of his career in ADC development, culminating in the approval of Kadcyla – the first ADC to receive full approval based on a randomized phase III trial. John is also a passionate rower and is Director of the Head of the Charles Regatta.

JIM MILLER

FOUNDER & PRESIDENT, PHARMASOURCE

Jim is a prolific speaker and writer, and an expert on pharmaceutical outsourcing. He is editor and publisher of two newsletters, Bio/Pharmaceutical Outsourcing Report and Emerging Markets Outsourcing Report. He served on the board of directors of the American Type Culture Collection from 2007 until 2016.



AJAZ S. HUSSAIN

PRESIDENT, NATIONAL INSTITUTE FOR PHARMACEUTICAL TECHNOLOGY & EDUCATION, INC., AND FOUNDER, ADVICE & SOLUTIONS LLC

“The FDA Science Board Discussion on the Emerging Issues in Pharmaceutical Manufacturing, November 16, 2001, was a pivotal moment for me because it affirmed my professional Vision 2020. This vision aims to transform the sector by improving assurance of quality and reliability of pharmaceutical manufacturing.”



ROMAN IVANOV

VICE-PRESIDENT R&D, INTERNATIONAL BUSINESS DEVELOPMENT, BIOCAD

Roman kindled his interest in cell biology as a schoolboy – taking part in Russia’s national cell biology “Olympic Games” and winning several international competitions. After completing his PhD in the now flourishing CAR-T cell field, Roman joined BIOCAD – then only a startup – which has grown into one of Russia’s leading biotechnology companies.



NICLAS NILSSON

HEAD OF R&D OPEN INNOVATION, LEO PHARMA

Niclas is spearheading an initiative to use truly open innovation at the core of drug research, with a focus on science and external collaborations. Prior to setting up the LEO Pharma Open Innovation platform, Niclas was heading the molecular pharmacology department. He says he has a passion

for pushing boundaries and is a strong believer of inter-disciplinary science. He is now exploring how to disrupt traditional partnering to promote mutually beneficial and more open ways to collaborate across barriers and borders. “Together we will boost innovation with opportunities that otherwise wouldn’t happen, and it starts by sharing. Freely moving science will unleash innovation to reach new heights that would benefit us all, young and old, rich and poor.”

DANIEL O'CONNOR

EXPERT MEDICAL ASSESSOR, UK
MEDICINES AND HEALTHCARE
PRODUCTS REGULATORY AGENCY

“It is extremely rewarding to be involved with regulatory pathways that promote earlier patient access to medicines in areas of high unmet medical need. Joining the MHRA from a clinical lecturer post was a complete change of direction in my medical career, but one that is dynamic, exciting and positively contributing to public health.”



DIANE PASKIET

DIRECTOR OF SCIENTIFIC AFFAIRS,
WEST PHARMACEUTICAL SERVICES

Diane has recently been working with the US Pharmacopeia on a revision of the elastomer monograph section for heavy metals. “The concerns for patient safety led to an international standard to assess and control elemental impurities in drug products (ICHQ3D Elemental Impurities) and a new USP monograph on extractable elemental impurities in elastomers will be introduced this year,” she says. Diane would like to see more industry collaborations and the advancement of data analytics. “This is a mission that involves an enormous accumulation of data; data correlation, integrity and proper handling is critical for providing value to patients.”



CORNELL STAMORAN

VICE PRESIDENT OF CORPORATE
STRATEGY, CATALENT PHARMA
SOLUTIONS

Cornell began his career as an accountant, before joining R.P. Scherer (now Catalent) in 1992. Cornell says he's always learning – and during his time at Catalent he has worked

across a number of areas, including strategy, marketing and press relations, innovation, investor relations, and mergers and acquisitions. Cornell also represented the interests of CDMOs as a member of the Pharma & Biopharma Outsourcing Association's generic industry GDUFA II reauthorization negotiation team.



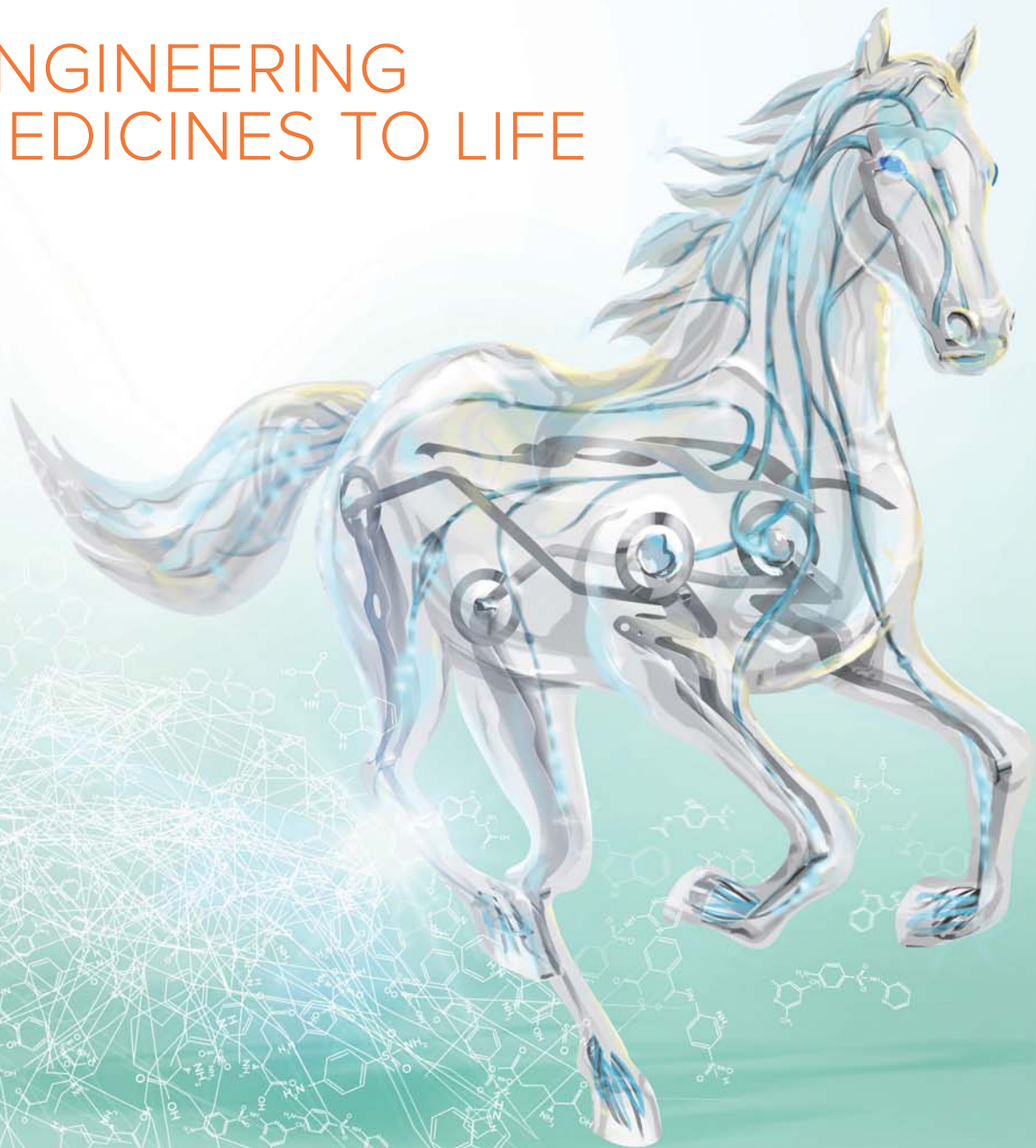
BERNHARDT L. TROUT

RAYMOND F. BADDOUR, SCD,
(1949) PROFESSOR OF CHEMICAL
ENGINEERING, MASSACHUSETTS
INSTITUTE OF TECHNOLOGY

In 2012, Bernhardt's team succeeded in running the first end-to-end fully integrated continuous manufacturing process. He would like to see “continued integration of functions, together with continued implementation of continuous manufacturing”.



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BUSINESS CAPTAINS

10. MANISH SOMAN



PRESIDENT AND CHIEF EXECUTIVE OFFICER, SCIFORMIX

“This year, Sciformix received the prestigious 2017 Global Pharmaceutical Scientific Process and Technology Outsourcing Customer Service Leadership Award from Frost & Sullivan. In addition, 2017 marks Sciformix’s 10-year anniversary. Since our inception as a small start-up headquartered in the US, we are now a global company with nearly 1,000 employees across the US, Europe, India and the Philippines.

“Technology has forever transformed the entire healthcare continuum and ushered in new and revolutionary ways to

ensure patient and consumer safety. To enable this evolution, one of the biggest challenges organizations face today is how to effectively integrate the complexities of two seemingly separate functions: the technology know-how behind IT, and the science behind safety operations.”

9. JOHN C. MARTIN

EXECUTIVE CHAIRMAN, GILEAD SCIENCES

John was appointed Executive Chairman of Gilead in March 2016 after a 20-year stint as the company’s CEO. John led the team that developed the blockbuster drug, Sovaldi (sofosbuvir), for the treatment of hepatitis C. He is due to receive the 2017 Biotechnology Heritage Award in June 2017.



8. HEATHER BRESCH

CHIEF EXECUTIVE OFFICER AND EXECUTIVE DIRECTOR, MYLAN

Heather has spent her entire pharma career at Mylan – starting as a data-entry clerk in the 1990s and working her way up to CEO in January 2012. She topped the Power List last year and has been included in Fortune magazine’s annual 50 Most Powerful Women in Business list since 2012.



7. RAMAN SINGH

PRESIDENT, MUNDIPHARMA ASIA PACIFIC, LATIN AMERICA, MIDDLE EAST AND AFRICA

During Raman’s six year tenure as President, Mundipharma has expanded from six countries to 128 and increased headcount from 350 to over 10,000. Mundipharma also won “Emerging Markets Company of the Year” at the 2016 Scrip Awards.



6. JEAN-PAUL CLOZEL

FOUNDER AND CHIEF EXECUTIVE OFFICER, ACTELION

After eleven years as a clinician, Jean-Paul decided to move to applied research. He spent 12 years at F. Hoffmann-La Roche, during which time he was responsible for the selection of the first T-channel blocker. He then founded Actelion in 1997, together with his wife and work colleagues and friends, and has since built Actelion from a start-up to a multi-billion market capitalization company.

5. SEVERIN SCHWAN

CHIEF EXECUTIVE OFFICER,
ROCHE GROUP

After completing his studies at the University of Innsbruck in Austria, Severin joined the Roche Group in 1993 as a trainee in corporate finance. Thirteen years later, he was appointed CEO of Roche's Diagnostics Division, and in 2008 he became CEO of the Roche Group.



2. SEUNGSUH (STANLEY) HONG

SENIOR ADVISOR, CELLTRION
HEALTHCARE CO. LTD

Stanley joined Celltrion in 2002 and was involved in the development of Remsima, the first biosimilar monoclonal antibody to be approved by the European Medicines Agency. He was President and CEO of Celltrion until December 2015.



4. BELEN GARIJO

CHIEF EXECUTIVE OFFICER,
HEALTHCARE, MERCK KGaA

Belén Garijo has been a member of the Executive Board of Merck since January 2015. She is responsible for the Healthcare business sector, comprising the Biopharma, Consumer Health, Allergopharma and Biosimilars businesses. Since 2013, she also acts as President and CEO of the Biopharma business, where she started in 2011 as Chief Operating Officer. Before moving to the pharma industry, she was a practicing physician.

1. JOSEPH JIMENEZ

CHIEF EXECUTIVE
OFFICER, NOVARTIS

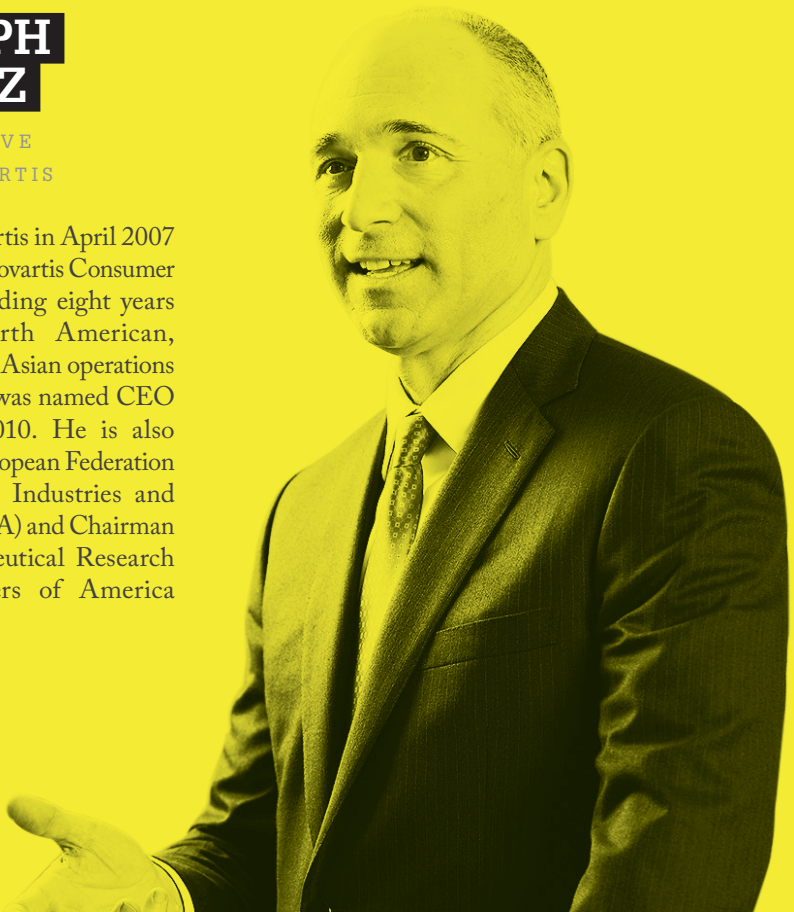
Joseph joined Novartis in April 2007 as Division Head, Novartis Consumer Health, after spending eight years running the North American, European and then Asian operations of H.J. Heinz. He was named CEO of Novartis in 2010. He is also President of the European Federation of Pharmaceutical Industries and Associations (EFPIA) and Chairman Elect of Pharmaceutical Research and Manufacturers of America (PhRMA).



3. KIRAN MAZUMDAR-SHAW

CHAIRPERSON AND MANAGING
DIRECTOR, BIOCON

From a tiny biotech start-up company launched in 1978, with a seed fund of 10,000 Indian Rupees and run from her garage, Kiran Mazumdar-Shaw has transformed Biocon into the largest publicly listed Indian biotech company. She is also known for her philanthropic work in the area of affordable healthcare delivery and she is the second Indian business leader to sign "The Giving Pledge." In 2016, Biocon made its debut on the Asia IP Elite 2016 list.



STEPHANE BOISSELCHIEF EXECUTIVE
OFFICER, TXCELL

“For many years, my field, cellular immunotherapy, was little more than a scientist’s dream – in fact, it only became a serious clinical option for patients a few years ago. But in 2017, it will likely become a commercial reality. Being involved in such a rapidly changing field is very exciting – especially when you know that patients’ lives are at stake. On a more down to earth level, the field of cellular immunotherapy, as with all innovative fields, brings new challenges almost every day. You have no time to rest, and only with passion can you maintain the required level of optimism and energy!”

**OLIVIER BRANDICOURT**

CHIEF EXECUTIVE OFFICER, SANOFI

Olivier has 28 years of global experience in the pharmaceutical industry, most recently as Chairman of the Board of Management of Bayer HealthCare AG and a member of the Executive Council of Bayer AG. He was appointed as CEO of Sanofi in February 2015.

**STEPHANE BANCEL**CHIEF EXECUTIVE OFFICER,
MODERNA THERAPEUTICS

Prior to Moderna, Stéphane was CEO of bioMérieux and he has also held leadership positions at Eli Lilly. This year, Stéphane revealed Moderna’s plans to develop vaccines for the most deadly strains of influenza, as well as a drug with the potential to treat heart failure.

JOHN CHIMINSKIPRESIDENT AND CHIEF EXECUTIVE
OFFICER, CATALENT

“We are honored that our customers put their patients’ needs directly in our hands. Though patients may not know us by name, they rely on our “patient first” culture. Our initial public offering in July 2014 was undoubtedly a pivotal moment in taking our organization to the next level – enabling us to bring more life-saving, patient-centric treatments to market.

“Across the industry as a whole, I would like to see continued commitment to developing treatments that demonstrate real-world results for patients. This could be fuelled by better understanding patient needs, more innovative drug delivery technologies, or through more collaboration – including helping our partners ‘rescue’ promising but difficult to deliver molecules.”

ROBERT A. BRADWAYCHAIRMAN AND CHIEF
EXECUTIVE OFFICER, AMGEN

In 2006, Robert joined Amgen as Vice President of Operations Strategy. He was appointed to the Amgen Board of Directors in October 2011, and became Chairman in January 2013, and CEO in May 2012. Before Amgen, he was a managing director at Morgan Stanley in London.





JOAQUIN DUATO

EXECUTIVE VICE PRESIDENT AND WORLDWIDE CHAIRMAN, PHARMACEUTICALS, JOHNSON AND JOHNSON

Joaquin has spent 27 years at Johnson and Johnson. He is also Chairman of the Pharmaceutical Research and Manufacturers of America, and was recently appointed to the Board of the CEO Roundtable on Cancer. In February 2017, he was named 2017 Honorable Mentor by the Healthcare Businesswomen's Association.

RODGER NOVAK

CHIEF EXECUTIVE OFFICER AND FOUNDER, CRISPR THERAPEUTICS

Rodger is one of the three co-founders of CRISPR Therapeutics, which aims to treat diseases using the breakthrough CRISPR/Cas9 gene editing technology. The company has recently been granted a patent for the technology by the European Patent Office.

JAMES C. MULLEN

CHIEF EXECUTIVE OFFICER, PATHEON

"During my time in the biopharma industry, much of my focus has been on finding the right people for the right roles who were driven to fulfill our mission of bringing products to patients that address high unmet medical needs. When I arrived at Patheon in 2011, I saw the opportunity to inject my perspective as a customer into the business. I believed redefining the relationship with our customers would change the way we were doing business and also transform the CDMO sector. Central to this

JANE GRIFFITHS

COMPANY GROUP CHAIRMAN, JANSSEN, EUROPE, MIDDLE EAST & AFRICA (EMEA)

In January 2011, Jane Griffiths became the first female Company Group Chairman of Janssen in EMEA, the pharmaceutical division of the Johnson & Johnson family. Jane says her personal mission is to strive for true diversity in her organization and beyond.



G. V. PRASAD

CO-CHAIRMAN AND CHIEF EXECUTIVE OFFICER, DR REDDY'S LABORATORIES

G. V. has been a member of the board of directors at Dr. Reddy's since the year it was founded. He joined in a full-time capacity in 1990, when company revenues were less than \$50 million – today they are over \$2.3 billion. Prasad is a strong believer in sustainable manufacturing and business practices, and is also involved with charitable initiatives including the Andhra Pradesh chapter of the Worldwide Fund for Nature and the "Acumen Fund," a non-profit venture that uses entrepreneurial approaches to help eliminate global poverty. He is also involved in helping to enhance higher education in India.



was attracting and retaining talent that embraced this commitment to our customers and engaged their teams to do the same. We were able to drive a lot of

change quickly, and today I'm proud that we are a company that can help customers simplify their supply chains with big ideas and solid execution."

RAFAAT RAHMANI

FOUNDER AND PRESIDENT,
LIFESCIENCE DYNAMICS

“Looking back now, the point where my career really took shape was when I was completing my MBA at Manchester University. The careers service published a dossier of all the MBA students’ CVs and circulated it. Eli Lilly & Company then came to our campus and called me for an interview. I had not thought about the pharma industry before, and in truth, I just went for the interview experience. However, I was lucky enough to be offered a job on the spot. I knew that pharmaceutical companies were very competitive and as I did not

have a background in that field, I was honored that they saw something in me. That interview with Eli Lilly set me on the path that would eventually lead me to start Lifescience Dynamics.”

**ABBE STEEL**

CHIEF EXECUTIVE
OFFICER, HEALTHIVIBE

Abbe is the Founder and CEO of HealthiVibe, a company that helps pharma companies gather patient insights to support clinical trial design throughout the entire product lifecycle. She has spent the past 27 years working in life sciences, leading patient initiatives for clinical development and post-marketing programs.

**PASCAL SORIOT**

EXECUTIVE DIRECTOR AND CHIEF
EXECUTIVE OFFICER, ASTRAZENECA

While at Roche, Pascal was in charge of integrating Genentech, after a \$47 billion takeover. He also led AstraZeneca in a successful defense against acquisition by Pfizer in 2014—a feat few envisaged before he took the reins. He initially worked as a vet before embarking on his career in business.

**IAN C. READ**

CHAIRMAN OF THE BOARD AND
CHIEF EXECUTIVE OFFICER, PFIZER

Ian began his career with Pfizer in 1978 as an operational auditor. He is a past Chairman of the Board of PhRMA and was recently elected the new President of the International Federation of Pharmaceutical Manufacturers & Associations.

MARTIN TOLAR

FOUNDER, PRESIDENT AND CHIEF
EXECUTIVE OFFICER, ALZHEON

During his academic career, Martin served as an Assistant Professor in the Department of Neurology at Yale University School of Medicine from 1992 to 1997, where he focused on movement disorders. Since then he has served as head of business development at Pfizer and more recently founded Alzheon, a clinical-stage biopharmaceutical company focused on brain health, memory and aging.



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Chemistry challenges
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CHAMPIONS OF CHANGE



10. SIR ANDREW DILLON

CHIEF EXECUTIVE, UK NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE (NICE)

Sir Andrew Dillon joined NICE as its founding Chief Executive in 1999 – and is known for his quiet determination. NICE has achieved much with him at the helm and many other countries look to the institute when deciding on their own drug policies.



9. ELISA CASCADE

PRESIDENT OF DATA SOLUTIONS, DRUGDEV

“I started at the University of Michigan, thinking I would go on to medical school, but I was so interested in economics that I ended up making it my major. After I graduated as a pre-med economics major, I realized healthcare consulting was a perfect fit for both of my interests and kept my options open for graduate training – business or medical school. That pivotal moment for me occurred when a mentor asked me ‘Do you like the idea of actually providing healthcare to patients or analyzing healthcare data?’ I thought long and hard about this, and being a closet data geek, ultimately chose healthcare data – I’ve loved working in this area ever since.”



8. DALVIR GILL

CHIEF EXECUTIVE OFFICER AND MEMBER OF THE BOARD OF DIRECTORS, TRANSCCELERATE BIOPHARMA

Dalvir Gill was appointed as CEO of TransCelerate in January 2013 and has since pioneered a number of changes and improvements to the drug development process. He has also been involved in launching BioCelerate, a subsidiary of TransCelerate, and its first initiative in Toxicology Data Sharing.



6. SÉGOLENE AYMÉ

EMERITUS DIRECTOR OF RESEARCH, FRENCH INSTITUTE OF HEALTH AND MEDICAL RESEARCH (INSERM)

Ségolène chairs the Topic Advisory Group on rare diseases at the World Health Organization and the European Union Committee of Experts on Rare Diseases. She is the founder of Orphanet, which provides information on rare diseases and orphan drugs.



7. CATHERINE TULEU

READER, UNIVERSITY COLLEGE LONDON SCHOOL OF PHARMACY

Catherine is also the Director of the UCL’s Centre for Pediatric Pharmacy Research. She has traditionally been interested in gastrointestinal drug delivery, particularly colonic targeting, but since 2003 her main focus has been on drug delivery systems for neonates, infants and children.



5. STEVE DAVIS

PRESIDENT AND CHIEF EXECUTIVE OFFICER, PATH; LECTURER ON SOCIAL INNOVATION, STANFORD GRADUATE SCHOOL OF BUSINESS

“A pivotal moment for me was living and working in Asia in the 80s – I spent time at a refugee camp in Thailand and I saw the havoc of the war in Southeast Asia playing out – it was graphic and it forced me out of my bubble. I have since used that visual when making

decisions, and the experience shaped my world view.

“I’d like to see increased access to sustainable innovation, especially in poorly functioning markets and low resource settings. During PATH’s 40 years of developing and delivering products, we’ve learned that when innovations fail to achieve widespread use, it is typically the result of interrelated issues at the global, national, community, individual, and partnership levels.”

4. ROBIN ROBINSON

RETIRED

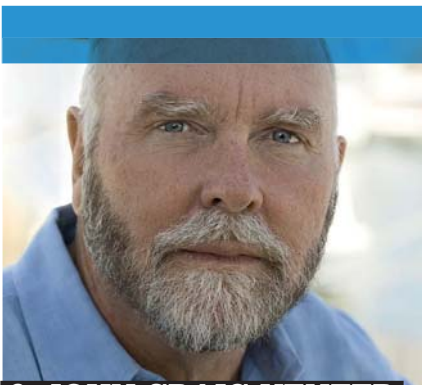
During his time in the pharma industry, Robin developed patented platform vaccine technologies including virus-like particles and subunit protein vaccines for human pathogens. Last year, he retired from the position of Director of Biomedical Advanced Research and Development Authority; and Deputy Assistant Secretary for Preparedness & Response, at the US Department of Health and Human Services.



3. ANTHONY S. FAUCI

DIRECTOR, US NATIONAL INSTITUTE OF ALLERGY AND INFECTIOUS DISEASES

Anthony has made seminal contributions to the understanding of how HIV destroys the body’s defenses leading to its susceptibility to deadly infections. In a 2017 analysis of Google Scholar citations, he ranked as the 21st most highly cited researcher of all time.



2. JOHN CRAIG VENTER

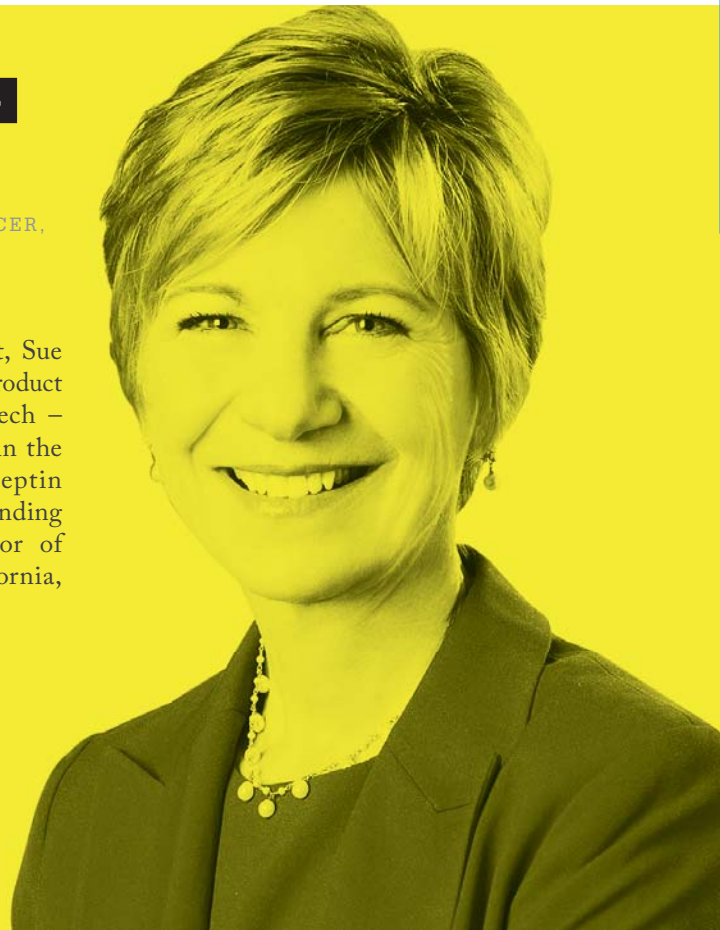
FOUNDER, CHAIRMAN AND CHIEF EXECUTIVE OFFICER, J. CRAIG VENTER INSTITUTE

John’s team created the first self-replicating bacterial cell constructed entirely with synthetic DNA. He is also the founder, scientific strategy advisor and executive chairman of Human Longevity, which seeks to use genomic data to tackle age-related diseases.

1. SUE DESMOND-HELLMAN

CHIEF EXECUTIVE OFFICER, BILL & MELINDA GATES FOUNDATION

Trained as an oncologist, Sue spent 14 years as head of product development at Genentech – where she played a role in the development of Herceptin and Avastin – before spending five years as Chancellor of the University of California, San Francisco.



CATHERINE BOLLARD

PRESIDENT, INTERNATIONAL SOCIETY OF CELLULAR THERAPY; PROFESSOR OF PAEDIATRICS, CHILDREN'S NATIONAL MEDICAL CENTRE

"In the 1980s, my best friend in high school, Diana, developed Hodgkin's lymphoma. Treatment comprised multiple cycles of chemo- and radiotherapy, but finally she went into remission. Later, she was diagnosed with myelodysplastic syndrome – a direct consequence of the Hodgkin's therapy – and died soon afterwards. It was so cruel, and it became clear to me that we needed therapies that kill malignant cells and not bystander cells. As a result, I became interested in cellular immunotherapy. I ended up working on Hodgkin's by chance when I



moved to Houston. We've now developed a T-cell therapy that gives complete remissions in over 50 percent of some patient groups, and two-year progression-free survival rates of over 90 percent in other groups."

**THOMAS CECH**

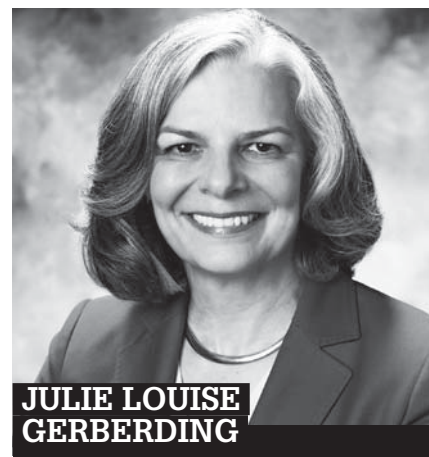
DISTINGUISHED PROFESSOR, UNIVERSITY OF COLORADO BOULDER

Thomas is a scientist with the Howard Hughes Medical Institute, and also a Distinguished Professor at the University of Colorado and Director of the university's BioFrontiers Institute. He shared a Nobel Prize in Chemistry for the discovery of the catalytic properties of RNA in 1989. He recently won Colorado Boulder's Hazel Barnes Prize.

**FRANCIS S. COLLINS**

DIRECTOR, US NATIONAL INSTITUTES OF HEALTH

Francis is the Director of the US National Institutes of Health, the largest supporter of biomedical research in the world. He has made landmark discoveries of disease genes and his leadership was integral to the international Human Genome Project.

**JULIE LOUISE GERBERDING**

EXECUTIVE VICE PRESIDENT & CHIEF PATIENT OFFICER, STRATEGIC COMMUNICATIONS, GLOBAL PUBLIC POLICY, & POPULATION HEALTH, MERCK, SHARP & DOHME

Julie served as Director of the US Centres for Disease Control and Prevention director from 2002 to 2009 before joining Merck, Sharp & Dohme in January 2010. Julie is responsible for the company's Foundation and "Merck for Mothers," a global program to prevent maternal mortality.

**LEROY (LEE) CRONIN**

REGIUS CHAIR OF CHEMISTRY, UNIVERSITY OF GLASGOW

"I believe that the digitization of chemistry is the key to making drugs cheaper, and to driving the discovery of other drugs and complex formulated products. I'd like to see a digital inventory of drugs, whereby all drugs that have been sold can be made available forever rather than the current process of drugs going 'out of production' – much like the way books used to go out of print before they were digitized."

BEN GOLDACRE

FOUNDER, ALLTRIALS; SENIOR CLINICAL RESEARCH FELLOW, NUFFIELD DEPARTMENT OF PRIMARY CARE HEALTH SCIENCES

Ben is a doctor, academic, writer, broadcaster and campaigner for greater transparency in the pharma industry. He is the co-founder of AllTrials, a project that advocates for all clinical trials being listed on an open registry.



BRIAN OVERSTREET

CO-FOUNDER AND PRESIDENT, ADVERA HEALTH ANALYTICS

Brian is the co-founder of Advera – which aims to improve patient safety and reduce systemic healthcare costs via the comprehensive analysis of real world outcomes data. Brian also co-founded Bruliam Wines, a boutique California winery, with his wife.



ANDREW LEES

FOUNDER AND SCIENTIFIC DIRECTOR, FINA BIOSOLUTIONS LLC; ASSOCIATE PROFESSOR OF MEDICINE, UNIVERSITY OF MARYLAND SCHOOL OF MEDICINE, CENTER FOR VACCINE DEVELOPMENT

“Alone in the lab, late at night, I was evaluating a possible polysaccharide activation reagent, CDAP. I combined polysaccharide, reagent, and then protein to make an instant gel – showing crosslinking had occurred. It was a true eureka moment and it changed my life. CDAP allowed me to develop some financial independence and scientific recognition. It gave me the confidence and ability to start a company with a ‘not for much profit’ approach – I am passionate about making conjugate vaccines affordable in order to give more people access to life-saving vaccines.”

ROS SMYTH

DIRECTOR, UCL GREAT ORMOND STREET INSTITUTE OF CHILD HEALTH

Ros is recognized as leading researcher in children’s health. She chairs the UK Committee on Safety of Medicine’s Working Group on Paediatric Medicines, and is Director of the UK Medicines for Children Research Network Co-ordinating Centre.



EDISON T. LIU

PRESIDENT AND CHIEF EXECUTIVE OFFICER, THE JACKSON LABORATORY

“There are perhaps two pivotal moments of my career. The first was when I decided to go into a basic science laboratory after my clinical training (in 1982) just to ‘see what it was like.’ Working in the lab of J. Michael Bishop at he University of California, San Francisco opened my eyes to powerful experimental systems in the then ‘new’ field of molecular oncology. The second was my time in Singapore as the Executive Director of the Genome Institute of Singapore, where I learned how fundamental sciences can be the foundation for the economic development of a country. Today, I am focused on understanding genetic complexity. It is clear that, with some exceptions, every gene mutation can be modulated by other genetic variations or epigenetic imprints, and that for every phenotype, multiple genes are involved in either the induction or the maintenance of that phenotype. Thus, measuring, understanding, and harnessing complexity is the key genetic challenge of this decade.”

**MIKE REA**

CHIEF EXECUTIVE OFFICER,
IDEA PHARMA

“The turning point in my career was when I resigned from a company that had just been acquired, for reasons of principle, and then found the opportunity to launch IDEA as a result. Today, my passion is helping pharma understand (and drive) the interplay between invention and innovation (what is discovered versus what is launched successfully), with the aim of helping more great medicines reach patients.”

**TOMASZ SABLINSKI**

CHIEF EXECUTIVE OFFICER AND
CO-FOUNDER, TRANSPARENCY
LIFE SCIENCES

According to Tomasz, meaningful progress in making new therapies available to patients will be driven by the convergence of modern technology and science. “The industry must realize that the digital revolution does apply to drug development. And in practice, companies must become quicker at adopting a digital approach to clinical research, otherwise, big and small technology players will make them irrelevant.”

MARK PAXTON

CHIEF EXECUTIVE OFFICER, RX-360

“Globalization has created many more opportunities for adulterated medicines to enter legitimate distribution channels – and I am passionate about securing those supply chains. My pharma career first launched at a small pharma manufacturer in Lexington, Kentucky, and then I was asked to join PhRMA in 2006 to develop and head their international regulatory affairs program, which was an amazing opportunity that set the stage for developing many long-lasting relationships with national regulatory authorities. I have also worked at the FDA, which was an amazing experience. Today, I head up Rx-360 – I have always strived to do something entrepreneurial, and this is it.”

**MATTHEW TODD**

ASSOCIATE PROFESSOR, THE
UNIVERSITY OF SYDNEY; FOUNDER,
OPEN SOURCE MALARIA

Matthew says he wants to see a “robust and large-scale trial of open source drug development as a competing model for the pharmaceutical industry.” The turning point in his career was the moment he realized that problems in science would be solved more quickly if the industry were more open with what it can’t do.

**STEPHEN J. UBL**

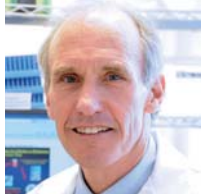
PRESIDENT AND CHIEF EXECUTIVE
OFFICER, PHRMA

Prior to his role at PhRMA, Stephen was president and CEO of the medical technology association, AdvaMed. He has also worked as vice president of legislation for the Federation of American Hospitals.

THE OVERALL TOP TEN OF 2017

For weeks, our esteemed judging panel has been sifting through nominations to decide who deserves a place in the 2017 Power List. Once the categories were compiled, we gave the panel a final challenging task... Here, based on their comments, we present the overall Top Ten Medicine Makers of 2017.

1. CARL JUNE



RICHARD W. VAGUE
PROFESSOR IN IMMUNOTHERAPY,
UNIVERSITY OF PENNSYLVANIA
[MASTERS OF THE BENCH](#)
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2. BELEN GARIJO



CHIEF EXECUTIVE OFFICER,
HEALTHCARE, MERCK KGaA
[BUSINESS CAPTAINS](#)
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3. ROBERT LANGER



INSTITUTE PROFESSOR,
MASSACHUSETTS INSTITUTE
OF TECHNOLOGY
[MASTERS OF THE BENCH](#)
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4. JOHN CRAIG VENTER



FOUNDER, CHAIRMAN AND CHIEF
EXECUTIVE OFFICER, J. CRAIG
VENTER INSTITUTE
[CHAMPIONS OF CHANGE](#)
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5. JOSEPH JIMENEZ



CHIEF EXECUTIVE
OFFICER, NOVARTIS
[BUSINESS CAPTAINS](#)
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6. NICHOLAS PEPPAS



PROFESSOR AND DIRECTOR OF THE
INSTITUTE FOR BIOMATERIALS, DRUG
DELIVERY AND REGENERATIVE MEDICINE,
THE UNIVERSITY OF TEXAS AT AUSTIN
[MASTERS OF THE BENCH](#)
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7. RICHARD JOHNSON



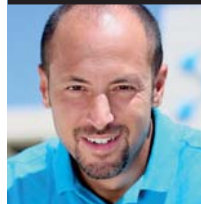
PRESIDENT AND CHIEF EXECUTIVE
OFFICER, PARENTERAL DRUG
ASSOCIATION
[INDUSTRY INFLUENCERS](#)
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8. KIRAN MAZUMDAR-SHAW



CHAIRPERSON AND MANAGING
DIRECTOR, BIOCON
[BUSINESS CAPTAINS](#)
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9. PHIL BARAN



DARLENE SHILEY PROFESSOR, THE
SCRIPPS RESEARCH INSTITUTE
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10. MARTIN VAN TRIESTE



CHAIRMAN OF THE BOARD,
PARENTERAL DRUG ASSOCIATION
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Ace Assessor

Sitting Down With... Daniel O'Connor,
Medical Assessor at the Medicines
and Healthcare products Regulatory
Agency (MHRA), UK.

How did you get involved with the MHRA?

I started my career in science and I've always been interested in translational research, particularly oncology. After my PhD I embarked on medical studies because I wanted to better understand the clinical implications of my work in cancer research. During that time I also worked as a post-doc and it was sometimes difficult to balance the science and medical school at the same time, but very useful to keep my hand in. Admittedly, I didn't get too much research done towards the end! After some junior jobs in the NHS, I became a clinical lecturer at UCL / Ludwig Institute for Cancer Research, which was exactly what I had always wanted to do – working on the translational aspects of clinical research. However, one day, I saw a job advertisement for a medical assessor position at the MHRA – and I was intrigued. I liked the idea of contributing to public health on a broader level. I got the job – and I've never looked back.

How did you find the leap from lecturer to regulation?

It was certainly a very steep learning curve! I had no regulatory experience and only a very top level view of drug development. But the agency is very supportive; mentors are assigned to new recruits to help them learn the ropes, and career development is encouraged. I started out as an associate and I have now been an expert assessor for a number of years. It is very gratifying to have reached this point after entering the field as a complete newbie! I really missed patient interaction and my research at first, but that changed as I joined various working parties, contributed to scientific advice and took on more complex new drug applications.

What qualities make a good assessor?

Attention to detail is crucial for effective evaluation of protocols or new medicine applications. A new product dossier may contain thousands of pages, and you have to

carefully review which data are critical and which are supplementary, and summarize your views in a concise and understandable report. You also need balance and the ability to make difficult decisions. Uncertainty is intrinsic to the regulatory approval process; you must be able to decide on what is an acceptable level of uncertainty, and that involves striking a balance between what data you'd ideally like to see and what you can live without.

How are you involved with the EMA?

I am the UK representative on the EMA Committee for Orphan Medicinal Products (COMP). I also sit on the Scientific Advice Working Party as a COMP representative, and on the Patient and Consumer Working Party I'm also an observer for the EMA Oncology working party. I find it very rewarding to be involved with EMA working parties and it is an excellent way of seeing and contributing to the latest, cutting edge pharmaceutical and regulatory developments.

In terms of my work with COMP, it has been exciting to see interest in the rare disease space increase dramatically. When I first joined the committee, we had maybe one and half days of meetings per month. Now we have packed-out three day meetings, which reflects the volume of new products being developed for orphan diseases. In part, orphan disease drug development has been aided by the fact that you can apply for orphan designation without clinical data, which is encouraging more academics to get involved in the field and interact with regulators.

What are your proudest achievements to date?

I was involved in setting up the MHRA's "Early Access to Medicines Scheme". I've always been passionate about patient access to new drugs, and the scheme has helped hundreds of patients to benefit from medicines prior to marketing authorization. It's very rewarding to make a difference

directly to people's lives as a medicines regulator with a desk job!

Secondly, on behalf of the Oncology Working Party, I was the lead on a new appendix guideline on patient-reported outcome measures in oncology studies published in 2016. This was the first new patient-reported outcome document since 2005, and I think a real watershed moment in terms of inclusion of patient-centric endpoints in regulatory decision making.

What big change would you like to see in the industry?

I would like to see better communication regarding the drug development process between different stakeholders. Education around the different aspects of drug development, as well as the respective roles of industry and regulators, would be particularly helpful. This would help balance some of the views regarding patient access and the public may better understand why it takes a long time for a drug to be developed and approved. More shared workshops between regulators, industry, patient groups and academics would also help dispel some of the myths and help to move difficult issues forward.

As I mentioned, I sit on the Patient Consumer Working Party, which is a great example of how to improve the engagement of regulators with patients and consumers. At the MHRA, we also think it's important to undertake public engagement activities, and have a patient consultative forum which is growing year on year.

Finally, I hope that as our understanding of disease improves we will see new therapies – not just new, advanced therapies, but also older drugs being repositioned and repurposed for different diseases. Orphan drug regulation encourages that kind of repurposing, which I think is really exciting. But advanced therapies will also be very important for the future – we are on the cusp of some very important developments in cell therapies and gene therapies over the next decade.



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