

INDUSTRY PERSPECTIVES

What Surprised You the Most This Past Year?: Your Colleague's Perspectives

Drug Development & Delivery's Global Formulation Report in this issue once again has provided our readers with highlighted (and notable) data on formulation and delivery, devices, fixed-dose combinations, transactions, and what's in the pipeline. We thought it would be interesting to ask some of your colleagues what they believe has surprised them most in the past year, and here is what they said!



"Advances in pharmacogenomics and biological therapies for 'personalized medicine' are in the process of revolutionizing the way we care for patients with cancer and many chronic diseases. Deciphering our human genomic make-up to administer tailor-made medicine for individual patients has unlimited exciting

implications for the future. The recent availability of flexible and modular filling equipment has been a key enabling technology for the transition of personalized medicine from R&D to commercial reality. Novel primary packaging solutions will also play a key role in advancing personalized therapies for the future."

- Chris Weikart, PhD, Chief Scientist, SiO2



"We were surprised by the enormous growth in the biopharma sector. We believe that as the importance of innovative pharmaceutical drugs increases, so will the importance of convenient and modern packaging solutions in glass and plastic. Especially, we developed the MultiShell vials and the GX RTF

Clearject syringes in COP because it's the perfect material to meet the challenges and properties of the new biopharmaceuticals. Suitable syringes and the right vials allow packaging to keep pace with these innovative drugs."

- Bernd Zeiss, Manager Technical Support Medical Systems, Business Development, Gerresheimer Medical Systems



"Over the past year, we have seen a surge of growth in the Pharma & Biotech industries. Our clients have been putting more emphasis on flexible design within their manufacturing suites. It has interesting to watch the architectural implications that are inherent to flexible design as well as single-use and continuous

manufacturing technologies. We have seen a reduction in the size of support spaces to manufacturing and opportunities to address material and personnel flows more efficiently within a facility."

- Christine A. Hofnagel, AIA, Senior Associate, JacobsWyper Architects



"A big surprise this year was a noticeable shift in emphasis from autologous to allogeneic therapies. There is a growing understanding that standardized, commercial-grade biologic products in commercial quantities must be manufactured at a reasonable cost so that they can be widely used. While this has been a

long-standing issue in the broader field of cell therapy, the change in emphasis is now evident in Japan's evolving approach to induced pluripotent stem cells, and in the growing efforts to manufacture allogeneic immunotherapies such as allogeneic CAR T-cell Immunotherapies."

- Karine Kleinhaus, MD, MPH, Divisional Vice President, North America, Pluristem Therapeutics



"The still relatively new gene-editing approach, CRISPR/Cas9, has proven to be a powerful tool and comparatively more attainable (easier to make, more cost-effective) research mechanism embraced by scientists across disciplines looking to modify gene structure. It has been impressive to see how rapidly this has moved toward human clinical

trials. University of Pennsylvania researchers achieved NIH Recombinant DNA Advisory Committee support for their proposed clinical trial using CRISPR/Cas9 as the next iteration of modifying T cells to fight cancer. Their hope is to attain FDA allowance and approval by their institution to initiate it in 2016, which would make them first in the US. Editas announced late last year their aim to be in the clinic sometime in 2017 with their CRISPR-mediated approach to treat Leber Congenital Amaurosis, an inherited retinal disease impacting children. We look forward to potential advances as U Penn and Editas clinical trials undergo FDA review as we progress our program utilizing CRISPR/Cas9 to treat patients with Fanconi Anemia; an inherited blood disorder impacting young people."

- Michelle Berg, Vice President, Patient Advocacy, Abeona Therapeutics



"As continuous manufacturing reaches new heights, the industry is adopting rigorous and robust measures to minimize risks and avoid batch failures. The excipient manufacturers are strengthening their capabilities to supply the high functional quality ingredients to help minimizing those risks and saving time and cost of drug

products. High functional excipients have been recognized for their utilities in multiple formulation technologies, conventional and non-conventional. As an example, the high functional excipient, such as copovidone (Kollidon® VA64), has been recognized as a gold standard in hot melt extrusion and in solid dispersion technologies, and on the other hand, it has been used as a dry binder in roller compaction and moisture-activated granulation and is also well suited to continuous manufacturing for increasing solubility of drugs and improving the tensile strength of the tablets."

- Shaukat Ali, PhD, Technical Support Manager, BASF



"The past year has seen a definite shift to the development of higher volume products for subcutaneous delivery. Many small biotech and large pharmaceutical companies' product pipelines for biologicals are focused on delivering higher doses, less frequent administration, or a move to subcutaneous delivery in the home

from intravenous delivery in a hospital setting. The ability to deliver higher volume subcutaneous therapies in a comfortable and convenient manner will become increasingly important to facilitate patient uptake and adherence to new therapies. Supporting the user with self-administration also holds great promise for reducing the burden of escalating healthcare costs."

- Michael Hooven, President & CEO, Enable Injections



"Over the last year, the development process for NMEs has continued to become more challenging, with the usual issues of bioavailability and safety being supplemented with other key hurdles, including dose form uniformity, oral delivery of macromolecules, targeted and controlled-release delivery, customer

and payer-friendly dose form development, scalability of the manufacturing process, and more. So just when we thought R&D complexity and productivity was tough, things got tougher. Thankfully, at the same time formulation technologies to enable solutions to these challenges have continued to advance, and drug development innovators have more options to get it right earlier, faster, and more efficiently if they plan early, think through their options, and partner with the right technology and formulation experts."

- Elliott Berger, Vice President, Global Marketing & Strategy (CMO), Catalent



"In the development of drug delivery systems for drugs and biologics, specifically combination products (CPs), it was most surprising to learn that despite the significant fanfare and reaction to the recent CP regulations issued by the FDA, there is a low perception within organizations that senior management is aware of the full extent and impact of device regulations on their internal drug/biologic development programs."

- Lilli Zakarija, MSME, MBA, Co-Founder & President, EdgeOne Medical Inc