October 15, 2018

Dear Regina,

Welcome to BioMarketing Insight's monthly newsletter.

Last month, I covered "Food Poisoning: Did People Forget the Seriousness of it and Customer Service?" If you missed last month's article, click here to read it. This month we'll cover " Highlights from Biotech Week, September 4 - 7, 2018"

Read on to learn more about this topic and other current news. The next newsletter will be published on November 15th, 2018.

We encourage you to share this newsletter with your colleagues by using the social media icons below, or by simply forwarding this newsletter or use the link below. Should you or your colleagues want to join my mailing list, click on the link below.

Sincerely,
Regina Au
Principal, New Product Planning/
Strategic Commercial Consultant
BioMarketing Insight

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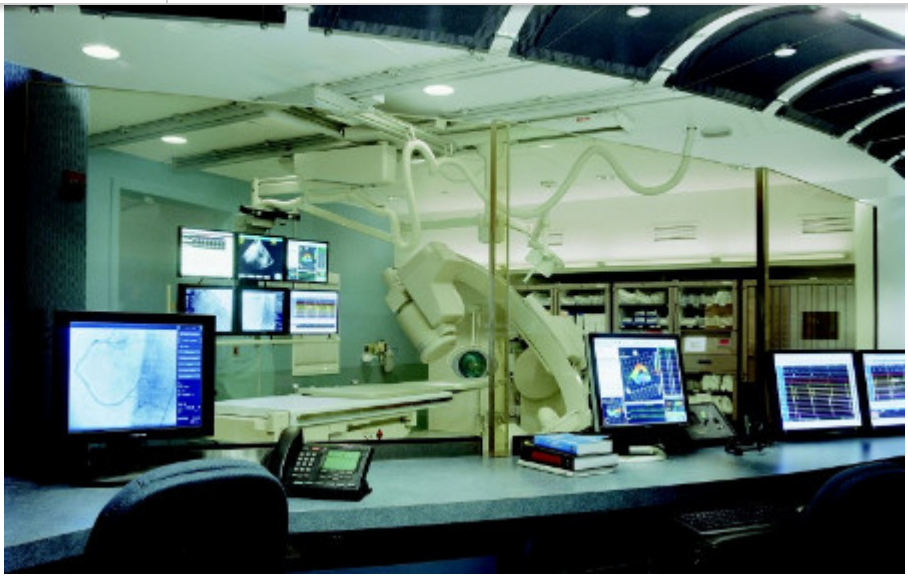

Developing a Product? Commercializing a Product?

If you are developing a product and have not conducted the business due diligence to determine commercial viability or success, contact me for an appointment. For successful commercial adoption of your product or looking to grow your business, contact me for an appointment.

For more information on our services, click on the links below:

Product Development
Market Development
Marketing Strategies

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I am pleased to announce that my article entitled "Updates in Solving the Mystery of Alzheimer's Disease Pathology" was published in the International Journal of Clinical Pharmacology & Pharmacotherapy. This commentary reviews the "Updated Proposed timeline of biomarker abnormalities leading to cognitive impairment" and the involvement of both beta amyloid clearance and plaque, and tau clearance and tau-mediated neuronal injury and dysfunction. To read the article, click here.

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Interview with Radio Entrepreneurs

I was privilege to be interviewed by Jeffery Davis, founder and co-host of Radio Entrepreneur, a radio station that shares the success of entrepreneurs. Click on this video to see a short clip of my interview.

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Why Our Microbiome is Important to Our Physiology and Diseases

I am pleased to announce that my article entitled "Why Our Microbiome is Important to Our Physiology and Diseases" was published in the International Journal of Clinical Pharmacology & Pharmacotherapy. This article reviews the results of the Human Microbiome Project and the factors that affect our microbiome in relation to our healthy state and dysbiosis or disease state. To read the article, click here.

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Immunooncology: Can the Right Chimeric Antigen Receptors T-Cell Design Be Made to Cure All Types of Cancers and Will It Be Covered?

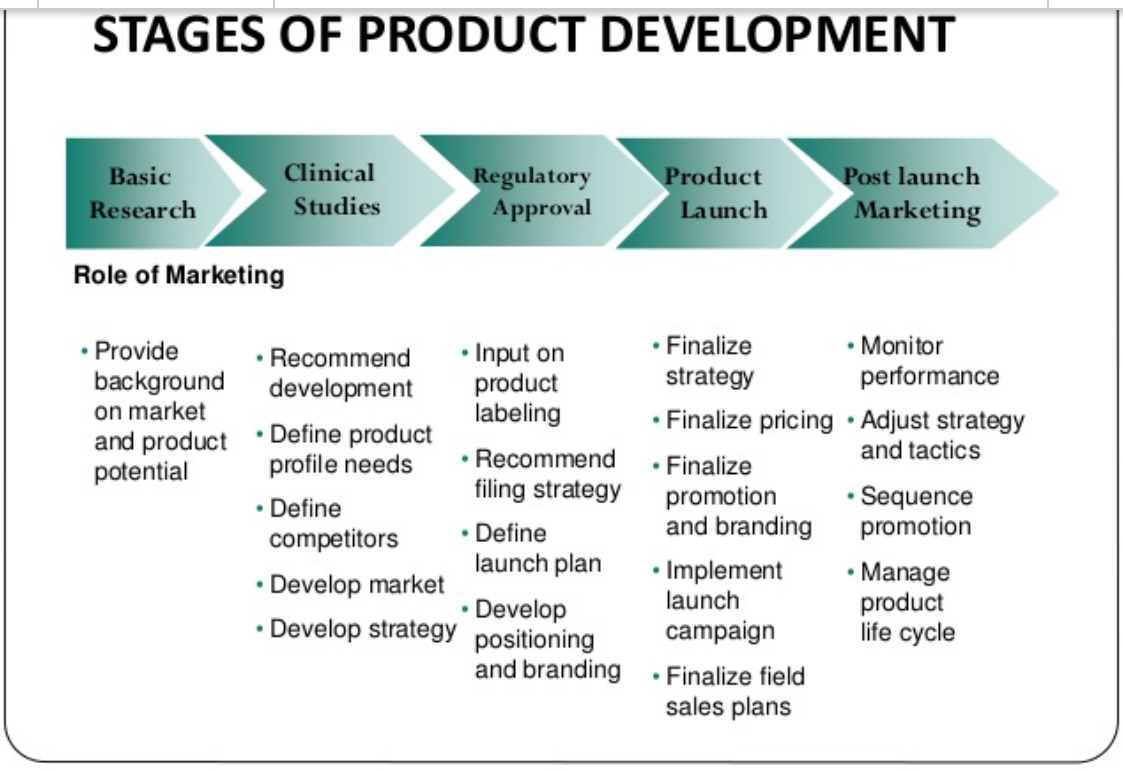
I am pleased to announce that my article on "Immunooncology: Can the Right Chimeric Antigen Receptors T-Cell (CAR-T) Design Be Made to Cure All Types of Cancers and Will It Be Covered?" has been published in Journal of Pharmaceutics. This article reviews the mechanism, design and administration of CAR-T cells, and whether payers will pay for this new technology. To read the article, click here.

Highlights from Biotech Week, September 4 - 7, 2018

Biotech Week comprised of a number of Biotech conference (BioProcess International, Cell & Gene Therapy, Microbiome Summit, AI Summit, BioPharm, Xcelerate and MassBio CRO/CMO Symposium) that were all held the week of September 4-7th at the Hynes Convention Center in Boston. All three floors of the Convention Center was devoted to the Biotech conferences.

The biggest conference in terms of attendees was the BioProcess International and the Cell and Gene Therapy conference which I attended and presented on "Why Speed to IND Can Cause Major Issues and Risks Later in Development." I also participated in a panel discussion on "The Downside to Speed to IND- Risks and Impact on Later Development" following my presentation.

My presentation "Why Speed to IND Can Cause Major Issues and Risks Later in Development" focused on why a company needs to include the business due diligence early in product development to derisk this process from the business side. In order for a company to be success, it required good business and science together.



Marketing/New product planning comes in as early as basic research to define the critical unmet medical needs and the product market potential as depicted in the diagram (high overview) above. This is by no means a complete picture of all the activities involved and the time line from a marketing role. This diagram is only used to indicate how early marketing comes in for the stages of product development.

Then marketing/new product planning comes in again once a company identifies a lead candidate, they still need to develop further to a Target Product Profile (TPP), this is your commercial profile. The product specifications of your TPP is determined by what the market dictates (includes market and technology trends) and of course your product has to be better because why would your customer buy it or insurance pay for it if it's not better. There are a number of reasons why this must be done before a company files for an IND and the consequences of not conducting it in my presentation. However, the scope of this newsletter is to give you highlights of the BioProcess and Cell & Gene Therapy conference, and for an overview of my presentation, click on my podcast below.

Why Speed to IND Can Cause Major Issues and Risks Later in Development

If you were unable to attend my presentation, here is a brief podcast interview with Dan Stanton from BPI on my presentation. To hear the podcast, click on my picture.

The panel discussion on "The Downside to Speed to IND - Risks and Impact on Later Development" included the Bioprocess perspective. However, for those who may not be familiar with the Bioprocess side, I will give a basic high overview and terminology since this is not my area of expertise and it's complex. In the product development process, these are the departments that are involved:

1. R&D
2. Marketing/Commercial
3. Regulatory
4. Clinical Development
5. Process Development/Manufacturing

In Process Development, it is divided up into two groups:

1) Upstream Processing- Bioprocessing begins upstream, most often with culturing of animal or microbial cells in a range of vessel types (such as bags or stirred tanks) using different controlled feeding, aerating, and process strategies.

This group is developing and growing the cell line to the required specifications or characteristics and generating a specific yield of product. The bigger the yield the better and validating it.

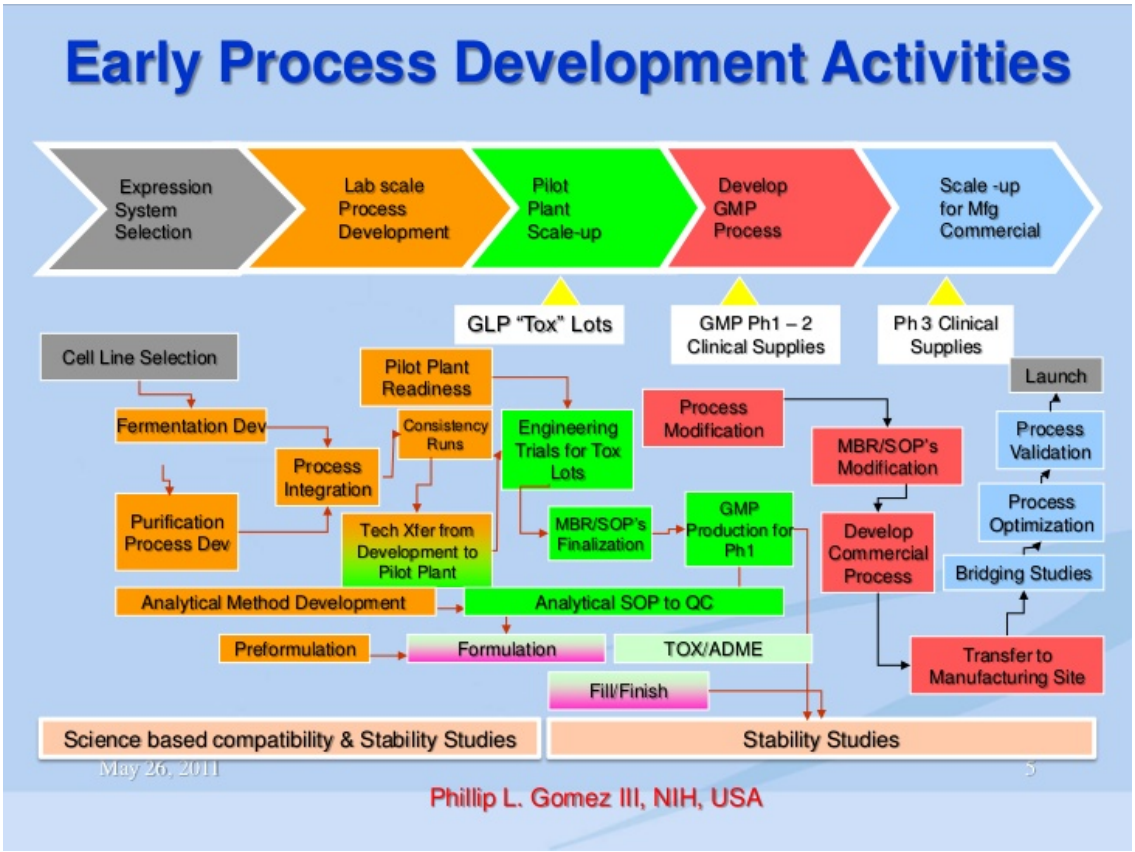
This usually involves cell culture media, perfusion cell cultures, assays and validation assays, expression platforms, and bioreactors.

2) Downstream Processing - Beginning with harvest of material from a bioreactor, downstream processing removes or reduces contaminants to acceptable levels through

This groups performs the purification process, confirming product yield and validating it. The diagram below shows a high overview or the process development.

This usually involves filtration, separation and purification, viral clearance and chromatography.

Needless to say, both upstream and downstream processing are both complex and complicated.



Because the process development activities are complex and complicated, rushing through the process in skipping steps saying it can be done later, or saying good enough will ultimately cause issues later in development particularly in your clinical trials and the data collected to prove that the product is safe and effective. It can also cause issues in manufacturing when the product needs to be scaled up if the yield is not properly calculated.

According to Leyla Diaz, Prinicipal Scientist at Millipore Sigma, she says that proper selection of your production cell clone is very important. In order for the clone to be well characterized, one must test the clone early in validating it rather than picking something and testing it later. For example, when one is producing antibodies, the following test must be performed: 1) binding; 2) good affinity; 3) stability; 4) half life in animals since

Conducting the assessment or testing early on, will save time later. For example, if one doesn't conducts the assessment early and only makes enough cells for example, Phase I clinical trial, the data may look fine for safety, but when one tries to scale up for the rest of the clinical trials, one may run into issues and/or the efficacy may be affected if the binding, affinity, stability or half life is inconsistent.

Leyla recommends that before one goes into clinical trials, have a robust process so that scaling up methodology is incorporated into the equation or process. Conduct as many engineering runs to test out the condition to achieve the optimal goal of the toxicology lot = your clinical lot. Allow sufficient time to develop the method for the correct phase of validation which usually consist of months instead of weeks.

There is a analytical method of validation for each phase of the clinical trial. And as product development continues, increasing emphasis is placed on identifying a stable, robust formulation from which multiple, bioequivalent lots can be manufactured and ultimately scaled-up, transferred, and controlled for commercial manufacture.

For example the purposes of Phase I is to determine a safe dosing range and key pharmacological data, typically in healthy human volunteers. The purposes of initial analytical methods are: 1) to ensure potency, that can be related directly to the requirement of a known dose; 2) to identify impurities (including degradation products) in the drug substance and product, that can be related to the drug's safety profile; and 3) to help evaluate key drug characteristics such as crystal form, drug release, and drug uniformity because these properties can compromise bioavailability. The FDA has guidance for Analytical Procedures and Methods Validation for Drugs and Biologics. To view those guidance, click here.

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Closing Thoughts

The process development activities are just as complex and complicated as R&D and marketing/new product planning. Therefore it is imperative that one takes the time to do it right up front for all three. For process development running the assessment or testing on the characterization of the product and production yield early on avoids issues and risks that impact development. This may also impact safety and efficacy, and whether the product will get approved by the FDA. It will save time in the long run as one avoids having to go back and starting over again or shutting down the program. Speed to market is important but not at the cost of eliminating important steps to ensure the success of the product in the market place.

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Should you have any questions or need of assistance with your business due diligence, determining your product's value proposition and economic value of your product, feel free to contact me at 781-935-1462 or regina@biomarketinginsight.com.
