Potential FDA regulatory changes with new commissioner – story of transition to Dr. Hahn from Dr. Gottlieb

Posted by By biotechwarrior March 4, 2021

There is a lot of speculation as to who will be the new commissioner of the FDA. With change in FDA commissioner, there usually is change in the direction of the FDA policies, among which the most important **one is its drug approval process.** Given the chatter in the market, I thought it would be interesting to discuss recent trend from the FDA under Dr. Hahn that appeared to be very different from FDA under Dr. Gottlieb.

As many biotech investors are aware, Biomarin ($BMRN) and Galapagos ($GLPG) received CRL from the FDA for Roctavian/valrox and filgotinib in August, extending review cycle for a long time – both events were HUGE surprises for both companies because EVERYONE expected those two drugs to be approved, including management teams who were super bullish on the launch of the drugs.

During earnings conference call, both companies mentioned that FDA's stance on their NDA / BLA applications has completely changed from FDA from pre-filing meeting (before NDA or BLA filing, companies sit down with the FDA to get guidance on the application – in this meeting, they usually feel out FDA's tone and proceed with filing if FDA sounds positive).

FDA is very conservative, not easy to understand and can be fickle with their decisions (or so many biotech CEOs say when they don't get approval the first time). However, events like Biomarin or Galapagos receiving CRL despite bullish management team comments do not happen very often – both are very experienced biotech companies with deep regulatory experience (in Galapagos, Gilead led the NDA filing process) – you would only see this in early stage companies without much experience in regulatory interaction ($HRTX received CRL twice and now they are trying to for the third try).

IT WAS A BIG SURPRISE FOR EVERYONE WHEN BMRN OR GALAPAGOS INSINUATED THAT CHANGE IN KEY LEADERSHIP POSITION AT THE FDA MAY HAVE PLAYED A PART IN WHAT IS ESSENTIALLY A REVERSE IN FDA'S VIEW ON FILABILITY OF INNOVATIVE DRUGS.

The "new leadership" that Biomarin and Galapagos mentioned is the transition of FDA commissioner to Dr. Stephen Hahn from Dr. Scott Gottlieb.

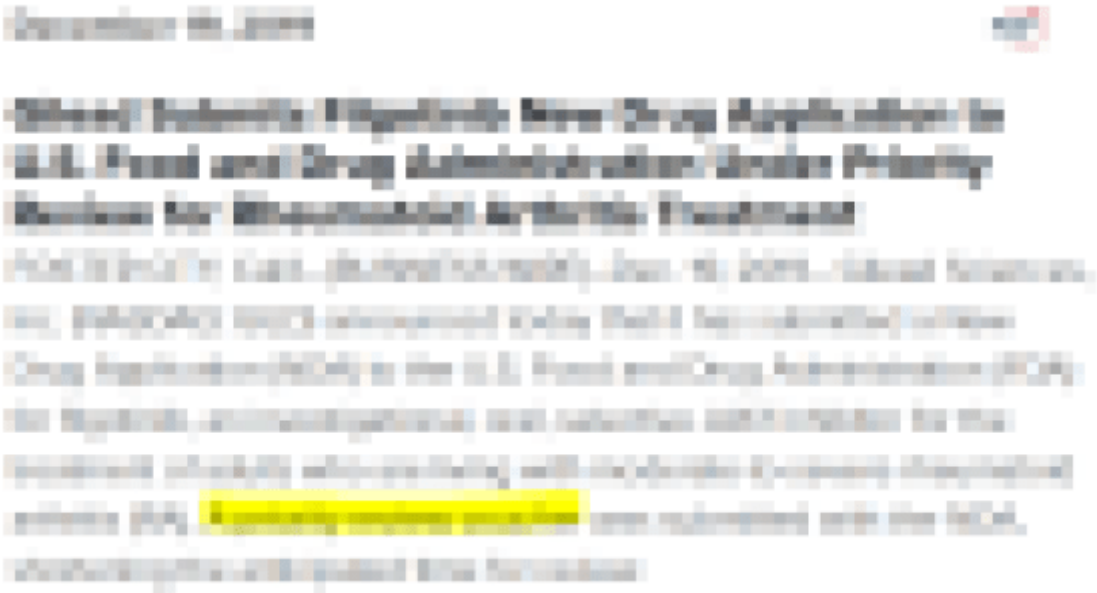
Prior FDA commissioner Scott Gottlieb was a very industry-friendly leader who also has working experience in venture capital. Under his leadership, the agency was considered very industry friendly – approving drugs with data whose quality may not have been good enough for approval under previous years and some criticized FDA for lower the bar. Their stance was clear – let's get innovative drugs fast to patients even though data may not be sufficient now.

On the contrary, Dr. Hahn comes from academia – this could mean that he is raising the bar back up, going back to very strict standard.

Interestingly, both Biomarin and Galapagos/Gilead had their pre-NDA / pre-BLA meeting before December 17th 2019 (before new commissioner became in charge), and they filed their NDA / BLA around the date of appointment.

Biomarin filed BLA for Roctavian on December 23rd 2019

Gilead/Galapagos filed NDA for filgotinib on December 19th 2019



Interestingly, aducanumab, an investigational therapy for alzheimers, had poor quality data, but FDA not only accepted the BLA, but also gave Biogen priority review for the drug.

With FDA's recent actions over the past 12 months, I think there are **three key takeaways.**

1. **NEW FDA COMMISSIONER LIKELY CHANGED DEPARTMENT HEADS OVER TIME AND THE CHANGES ARE FINALLY HAVING LARGE IMPACT** – BIG IMPACTS APPEAR TO HAVE BEEN IN NEUROSCIENCE, RHEUMATOLOGY, HEMATOLOGY.
2. **FOR DRUG APPLICATIONS THAT WERE BASED ON PRE-NDA MEETING UNDER PRIOR LEADERSHIP, IT MIGHT MAKE SENSE TO DOUBLE-CHECK THE PROBABILITY OF APPROVAL** – FDA MIGHT FEEL VERY DIFFERENTLY ABOUT THE APPROVABILITY NOW VS. THEN.
3. **DO NOT TRUST DRUG MANAGEMENT TEAM TOO MUCH** – SOMETIMES THEY JUST DON'T KNOW. GILEAD/BIOMARIN HAVE DEEP EXPERTISE IN REGULATORY AFFAIRS (I.E. THEY HAVE GREAT RELATIONSHIP WITH THE FDA), BUT THEY ALSO COMPLETELY DROPPED THE BALL BECAUSE THEY COULD NOT FEEL OUT THE CHANGE IN FDA'S THOUGHTS DESPITE MANY MEETINGS.

Healthcare is a sector with heavy regulatory risk and fickleness of FDA is only making it more difficult. In investing, it is always most important to not lose money. If you have stocks in companies whose regulatory result is due, please take a moment to consider above factors!