Optimal Strategix Group

Optimal Strategix Group, Inc.
140 Terry Drive, Suite 118
Newtown, PA 18940
+1 215 867 1880
www.optimalstrategix.com

Top 10 Signs that Next Generation Patient-Controlled Clinical Trials are Going Mainstream

By GIRI IYER, EVP & GM, Analytics, OSG dated 5/18/2015

The Problem: Well understood in the industry circles, it is a well-documented fact that clinical trial costs as a percentage of drug R&D costs are now hovering around 90% for approved drugs. Recently Matthew Herper, Forbes estimated in his 2013 article that the average cost had exceeded $5 billion per drug [1]. Yes, $5 billion! Little surprise then that the average cost per drug for patients is on the rise and global access to cutting-edge medicine is becoming a more uphill challenge. The average drug takes 10-12 years or more to bring to market largely due to the rising complexity of clinical trials. The human cost we bear due to the process bureaucracy and inefficiencies are telling. According to some experts, an average of 35,000 patients die waiting for potentially viable drugs to move through the clinical trial process [2]. The FDA and the EMA have both made significant strides towards regulatory reform including the ability to fast-track life-saving drugs with clear chances to extend lives of patients and showing early promise [3]. However one thing that has not changed yet is the role of the patient in the process. For too long we have expected the patient to adapt their lives around the scientific rigor and procedural complexity of the clinical trial process. According to Tufts Center, leading experts in clinical trial economics, patient enrollment fell 21% and patient drop outs increased by 30% in the period 1999-2005 [4]. Recent surveys by EyeforPharma indicate that 42% of Pharma executives would like the speed of clinical trial enrollment to accelerate [5].

The Hope: Let us acknowledge some tremendous work by The Clinton Foundation [6] and the Gates Foundation [7] are both doing some tremendous work in getting life-saving drugs to the parts of the world that are in dire need of simple life savings drugs to fight malaria, HIV/AIDS, Tuberculosis, etc. We also know that research and regulatory organizations are getting together to create collaborative frameworks that accelerate trials. We have seen thought leadership and initiative from Pharma companies like Pfizer [8] and Sanofi [9] and clearly see the industry moving in this direction. However, as with any new innovation, one encounters the risks and the inevitable failures for simply daring to try something new. We salute the pioneers and join them as soon as early signs of progress that something is here to stay. When will we know we are seeing the next wave of clinical trials emerging with faster, cheaper, better outcomes?

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Top 10 signs that we are entering the era of patient-controlled clinical trials

1. **@Home trials are a reality**: The clinical trial process is now designed around the patient and we begin to hear that most patients can do entire clinical trials from their home. Most of the patient reporting is done through simple, easy applications that take just a few minutes for the patient. It is only the real complex trials for high acuity patients that require nurses to visit the patient's home. Look for the first 100% @home trial conducted entirely on-line between the investigator and the cohorts for a simple low or medium acuity trial to succeed.

2. **Digital Health is mainstreaming**: The Internet of things (IoT) revolution has seen the advent of smart weighing machines, smart phones, smart sensors and wearables. All of these devices, sensors and tools are aimed at one thing: Eliminating data entry for the patients. Look increasingly for applications that use Apple, Google or Microsoft mobile platforms to automatically collect as much information about physical activity, sleep, heart rate, temperature, etc. to be submitted to the trial investigator. This is a patient delight and while 100% automation is elusive, look for patient satisfaction increase of 50% to be the tipping point measure that we are past the tipping point of this revolution.

3. **Patient Electronic Consent is the norm**: We are going to see this happen rapidly due to the new regulations around patient consent on-line using biometrics or even simple digital signatures. The long patient consent form will fast become a thing of the past with 100% conversion a real milestone soon. Among the top ten signs, this one is the low hanging fruit that will happen fastest.

4. **Patient recruitment goes on-line**: This revolution is the beginning of the virtual clinical trial process. We have seen industries adopt on-line registration and on-line matching with great success. From Expedia and Travelocity in travel to hotels.com in hospitality to Fandango in entertainment to Amazon and EBay in ecommerce, we have seen industries go online and fast in the last decade. While travel agencies have not gone away, the real benefits we gained as customers was easy global access and ease of transacting. Travel agencies had to offer value added services to differentiate and evolved. Expect the same to happen in the clinical trial space with >50% of all recruitment going online in the next five years as the true mainstreaming of this mode of patient recruitment.

5. **Data Privacy rules are recalibrated**: We are now in the midst of a new global generation of millenials who have a different sense of privacy than we do. They

share so much more as netizens with each other every day than we are ever comfortable doing. These millenials are grand children and children of patients and often are part of the care team pushing their parents and grand parents to do more to take care of themselves including finding new trials for them. They are the ones pushing across generational barriers to get their loved ones to put more information out there to accelerate the chances of getting into the trial and even to keep them there. We share so much financial and personal information already online so expect the reservations to relax quickly in this new revolution. Of course, patient informed consent is the magic wand that makes this revolution happen, not policy, not technology, its patient power. All they have to do is get comfortable sharing some information online to make this revolution a reality after fully understanding the risks and benefits and making an informed choice to engage. We know from the many private patient communities that this is not something to bet against. It's only a matter of a few months and we will see this barrier fall.

6. **Social media angst is overcome:** One of the biggest assets that enable new thinking is the vibrant global social community that is self-organizing and self-managing. Many of these are well known groups of disease advocates, patient advocates and other birds of a feather using Facebook, Twitter, and other social forums to organize and mobilize support. They also represent the biggest risk and worry to the purists since these online communities and individual patients could potentially contact each other and decipher who is on a placebo and who is not by sharing information with each other based on how they feel, other effects, symptoms, etc. One might argue this is happening today already despite all the safeguards in the current legacy process. Notwithstanding this, we will need patients to respect the scientific cause and purpose of the trial so they do not sacrifice the integrity of a trial by accidentally or deliberately introducing bias into the system. Expect to see code of conduct and eBay-like ratings of patients based on how well they behave as participants in this important process. For the new process to succeed, we need to establish digital trust at scale, something again we know works for >99% of all online transactions today.

7. **Research integrity remains uncompromised***:* One of the biggest worries of researchers is the integrity of the study gets compromised due to the transformation of the study to cede some level of control to the patient. A double-blind study with multiple arms includes careful preparation, planning, data collection, analysis and reporting based on cohorts randomized to eliminate bias. We will need software systems that understand the integrity of this process so the clinical trial remains objective, unbiased and uncompromised in its scientific

integrity. Researchers and clinical teams bring years of expertise to the table today across multiple sites globally to achieve this. Emulating this scientific integrity in a patient-controlled paradigm is non-trivial and patient education will play a critical role to achieve this level of research integrity with no compromises. Amongst the skeptics, this is the litmus test of mainstreaming that we will have to fight hardest to overcome.

8. **Regulatory support**: While the FDA, the EMA and others are clearing sending signs of supporting change; they also carry a tremendous fiduciary burden to protect the citizens of the regions they serve. Their burden is heavy since every mistake of the regulatory system can be headline news and political and social scrutiny is hard and unrelenting. They need to be reassured that while the industry move to the new patient-controlled trial frontier, we are being more transparent with our data and sharing success and failure with the regulators objectively. Discerning signal from noise coming from the world of social media can be harder than it seems since individual responses from patients often lack the objectivity and can dilute from the real outcome.

9. **Favorable trial economics overcome most objections**: No industry has survived the onslaught of the web and with Internet 3.0 upon us we are now in the generation of the Uber and the Airbnb economy. Physical rigid infrastructures are being overlaid with social collaborative infrastructures that are ad-hoc and spontaneous. The savings are real and the consumer satisfaction and convenience are up. Patient-controlled clinical trials are no exception to this rule and we will see Pharma and Medical Device Company demonstrate significant financial value to their R&D investments and shareholders based on this transition. A 10% saving on a trial can save $500 million to a Pharma and help fund five exploratory studies for compounds inside a major Pharma company. This will unleash innovation as well for smaller Pharma and Biologic start-ups.

10. **Behavioral Analytics to the fore:** Yes, we have been too clinical for our own good. We even named these research studies clinical trials. And yet barely 10% of these trials finish on time because patients don't demonstrate good compliance? We need to add behavioral test and profiles to the recruitment profiles so they are more than just clinical inclusion and exclusion criteria. We need to secure patient engagement and close the loop with patient reported outcomes. In this era of Precision Medicine, we need differential diagnostics and differential benefits in order to secure drug viability. Comparative Effectiveness is a difficult exercise to demonstrate particularly if we use only financial metrics.

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Companies with proven platforms and pedigree in behavioral and cognitive analytics will blend next generation patient behavior modeling and stratification with new types of patient reported outcomes. Behavioral analytics will become useful part of the primary benefits or at least secondary benefits that are touted by drug companies in order to secure payors reimbursement and/or marketing effectiveness. Call this the Holy Grail, but we will include behavioral criteria in 50% of the trials sooner than later. This is not because it is a novelty but because it secures better patient engagement, helps recruit patients faster and reduces patient attrition. It accelerates drug time-to-market and improves returns on R&D. A 1% reduction in patient attrition during trials can save a Pharma company $5M and this will accelerate the time to market by 5-10 days.

Moving Forward

We hope this information has been interesting and valuable to you. Please, feel free to share it with colleagues and other people in your network. We look forward to start a dialog with you about this topic and share more information about our knowledge and experience in this field.

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About the Author: Giri Iyer has spent over two decades at the intersection of technology and business. He has 15+ years of experience in Medical Imaging and Healthcare Informatics from Siemens, Philips, GE Healthcare IT and now OSG. He is today the Executive Vice President & General Manager, Analytics at OSG and can be reached at giri.iyer@optimalstrategix.com with your critique, feedback and suggestions on how to accelerate this change in our industry.

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