A biotechnology company developed a new treatment for metastatic breast cancer but was faced with the challenge of a major registration trial in an area where competition for patients was fierce. The product was felt to have a powerful market potential because of its ability to improve tumor response rate and reduce side effects. In addition, the product was the company’s flagship product, but development resources were guarded. Failure of a major product like this would be a major blow to the company as well as threaten its viability.

**Key Challenges**

- **Enrolling in a difficult therapeutic area**
  Enrollment in oncology studies is often challenging, especially in the crowded U.S. market. Despite this obstacle, the sponsor strongly preferred to conduct the study in the U.S. while still recognizing the need to enroll the study as rapidly as possible.

- **Minimizing development costs**
  The resource constraints were even more pointed for this company because of the magnitude and cost of a major registration study and the consequences of failure. The study had to achieve rapid enrollment and accurate, reliable results without breaking the budget and without cost overruns.

- **Accelerating generation of revenues by the sponsor’s lead**
  The sponsor had to rely on investors to continue funding its burn rate until revenues started coming in so it was important to reduce development timelines to get to market faster.
Solutions

- **Adaptive Design: Sample-Size Reestimation**
  This technique, designed and negotiated by Health Decisions and the first FDA-approved adaptive study for oncology was used to adjust sample size midway through the study. This measure allowed the sample size to be reduced by more than 50 patients because of better-than-expected efficacy. Combined with other Adaptive Operational practices, these adjustments enabled the study duration to be shortened by nine months.

- **Adaptive Operations: Tiered enrollment strategy**
  Within two months of the study start Health Decisions’ team learned that enrollment via U.S. sites alone would not be able to meet the sponsor’s aggressive timeline. The team rapidly expanded the study to a second tier of prequalified sites in Canada, the UK and Russia. This tiered strategy, an Adaptive Enrollment technique, saved three months vs. planned timeline and was the fastest enrolling mBrCa study found in literature.

- **Adaptive Operations: Optimized allocation of resources**
  Health Decisions’ proprietary reporting platform provided site performance metrics, within hours of patient visits, to identify and correct problems to ensure optimal use of the resources. Ultimately Adaptive Monitoring drastically reduced error rates and improved data quality.

Financial Impact

In the end, three key adaptive elements – sample-size reestimation, adaptive enrollment and adaptive monitoring – allowed Health Decisions to save the sponsor a full year of development time. SSRE primarily saved nine months and the tiered enrollment strategy saved three months. This combination of adaptive design and adaptive operational practices saved the sponsor more than $2 M in development costs and got the product to market a year ahead of schedule.

An analyst estimated the total value created by Health Decisions having brought this product to market a year early was in the order of $1 B. With first year sales of $366 M and an extra year of patent protection, the estimated market size for this product in 2015 is $1 B.

*Nine months saved through sample-size reestimation, three through rapid enrollment.

**From sample-size reestimation and shortened timelines.

***From extra year of marketing under patent protection and acquiring company’s forecast of sales.

The Health Decisions Difference

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<tr>
<th>SPONSOR BENEFIT</th>
<th>1</th>
<th>$2 M</th>
<th>$3 B</th>
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<tr>
<td>TIME SAVINGS (YEARS)*</td>
<td>DEVELOPMENT COST SAVINGS**</td>
<td>ADDITIONAL REVENUE***</td>
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About Health Decisions

Health Decisions is an innovative CRO that for 25 years has enabled forward-looking biopharma and device companies to bring products to market successfully, earlier and with less risk. Notable successes in IVD studies include early completion of both a 4,000-subject study of a diagnostic for human papilloma virus and a 13,000-subject study of a diagnostic for colorectal cancer. These large IVD studies illustrate how Health Decisions’ market-leading Agile Development methodology, powered by LiveData™ and advanced analytics, enables highly trained and experienced project teams to provide responsive, proactive trial management that consistently meets or exceeds development goals for sponsors worldwide.

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