

"Orphan Drugs": Hope Where There Is Little or No Hope

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By Alex Michelini

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NEW YORK, N.Y., February 18, 2004 - On a visit to his doctor, Gary Jacob received distressing news - not about himself, but a friend of the doctor's.

While playing with one of his children, the doctor's friend fell and broke a rib. That was bad enough, but during the examination at the hospital, the father was hit with a startling and totally unexpected diagnosis - he had a disease known as multiple myeloma, a bone marrow blood cancer.

The diagnosis was nothing less than a death sentence.

Jacob knew of the anguish of multiple myeloma patients. The disease is incurable and nearly always fatal, one of the rare diseases that have few, if any, available treatments. They are known as "orphan" diseases, shunned by most drug-makers because the patient populations are small and commercial development of a drug is seen as economically unattractive.

Mr. Jacob was aware because, as Chief Executive Officer of Callisto Pharmaceuticals, Inc., a small Manhattan-based biopharmaceutical company, he is leading a scientific effort to develop a new orphan drug called "Atiprimod" for multiple myeloma patients.

"The father's disease brought home to me that what we are doing is really important," says Mr. Jacob. "Everyone agrees we need more drugs to treat multiple myeloma. There are people out there dying without real hope because of a lack of effective treatment for all patients."

In steadily increasing numbers, orphan drugs are providing new doses of hope where little or none at all existed. In the decade before the inception of the federal Food and Drug Administration's orphan drug program, 10 drugs were developed by pharmaceutical companies for orphan diseases. In the decades since, the FDA says nearly 250 new drugs were developed and approved, and hundreds more are in the pipeline.

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Atiprimod is one of those wending its way toward the marketplace. Callisto recently obtained orphan drug designation from the FDA, providing the company with financial incentives to continue the costly development process.

The program covers drugs for orphan diseases with patient populations under 200,000.

The National Organization for Rare Disorders reports about 25 million people in the United States suffer from an estimated 6,000 orphan diseases.

Diseases such as cystic fibrosis, complications affecting HIV–infected people, Gaucher's disease, hemophilia and rare forms of cancer were among the orphans without effective medicines until the FDA program went into effect in 1983 and paved the way for new drugs for patients with these diseases.

Large drug–makers have been largely missing from the efforts.

According to the orphan drug program's deputy director, Dr. John McCormick, only 15% of applications for orphan drug designation have come from the larger pharmaceutical companies.

The reason: expectations of unfavorable investment returns.

The FDA orphan drug incentives - grants, seven years of marketing exclusivity and tax breaks - have drawn small pharmaceutical companies with promising drug candidates into the breach.

While the future is brighter, the task is still daunting to develop drugs for orphan diseases.

Amyotrophic lateral sclerosis (ALS), or Lou Gehrig's disease, affects 30,000 Americans with 8,000 new cases diagnosed annually; Huntington's disease also affects about 30,000 patients.

Some diseases affect fewer than 100 patients, according to the National Institutes of Health.

An estimated 50,000 patients have multiple myeloma with 15,000 new patients diagnosed each year. Last year, the FDA approved a new drug Velcade for patients with the disease. However, there are still a number of multiple myeloma patients with no treatment available.

Dr. Kenneth C. Anderson, who played a major role in the preclinical development and clinical trials of Velcade and is now a member of Callisto's Medical Advisory Board, is among the experts who see a need for more drugs to treat multiple myeloma.

"He is excited to see Atiprimod enter clinical trials for evaluation in multiple myeloma patients," Jacob said of Anderson. "He believes it has an opportunity to help patients who have not responded to other drugs. "

Dr. Anderson is director of the Jerome Lipper Multiple Myeloma Center of the Dana–Farber Cancer Institute in Boston, MA, and Professor of Medicine at Harvard Medical School.

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The Phase I/IIa trials for Atiprimod are slated to begin later this month.

Dr. Donald Picker, Callisto's Senior Vice President of Drug Development, said studies of Atiprimod in collaboration with scientists at the National Cancer Institute have been very promising.

"In essence, we've shown in these early studies that Atiprimod has the potential to intervene with cancer cells and tumors in three ways - by inhibiting their formation, by programming their death and by limiting their ability to grow blood vessels necessary for their survival. Taken together, these findings suggest that Atiprimod could potentially represent a novel class of compounds for development for therapeutic intervention in human cancers," said Dr. Picker.

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Alex Michelini is a former award-winning reporter/editor at the New York Daily News. His credits include nomination for a Pulitzer Prize for a series on medical costs. Among his honors, Mr. Michelini received the Deadline Club Award, the Page One Award, the Associated Press Award and the New York Press Club Award (twice). He is the founder of Alex Michelini Public Relations, and has developed and collaborated on articles appearing in the New York Times, the Wall Street Journal, the London Times, the New York Post, the New York Daily News, Bloomberg Radio & TV, CNNfn, WCBS Radio & TV, WINS radio, the Christian Science Monitor, Fox TV, Reuters, Newsday and other media outlets.

Beyond Hope is Faith

By Jean R. Charles

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I live in a small town named after its Dutch settler, whose name is long and difficult to spell and pronounce. The town just to its south is named Hope, and is well known because of its restored historical status. So, when residents of our town are asked where they live their frequent response is "I live beyond Hope".

Recently, as I was driving home beyond Hope, I began thinking about what it really means to live beyond hope. Hope is about wishing for something to occur in the future. It implies uncertainty and longing. You want something and do not know if you will get it.

Beyond this future oriented wishful thinking is faith. Faith implies a knowing. It involves trusting the universe to provide. When you live in faith you intuitively know that things will happen as they are meant to happen. You realize you are where you are and have what you have for a reason. You are satisfied not knowing the answers to the why questions.

Hope is a beautiful little town and a great place to live. However, spiritually, living beyond hope, living in faith is more rewarding and peaceful. Perhaps we should consider changing the name of our town to

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Faith. This would fit well with the beyond Hope thing, and also with our other neighboring towns of Harmony and Tranquility, but those are other stories.

"Faith sees the invisible, feels the intangible and achieves the impossible." Anonymous.

Jean coaches professionals and small business owners to discover, design and live their just right lives. She can be reached at jean@justrightcoach.com or visited on the web at www.justrightcoach.com. Subscribe to Just Right Coaching Clips newsletter by sending a blank email to justrightcoaching-subscribe@topica.com

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