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Personalized medicine continues to explode, especially against cancer

By ACSH Staff — May 8, 2015

We have seen some remarkable medical breakthroughs in the past two decades. In terms of sheer impact, it could be argued that transforming HIV infection from a certain death sentence into a manageable chronic disease, and a cure for hepatitis C, which is four times more prevalent than HIV worldwide, are at or near the top of this list.

Although progress against certain cancers has been significant (and more newsworthy), many cancers remain untreatable, or, at the very least barely treatable.

This is evident from a list [1] of approved cancer drugs in the past two decades. Some of these therapies have resulted in cures, or significant extension of life, but as ACSH s Dr. Josh Bloom pointed out in his 2013 New York Post op-ed [2], many of the newly approved cancer drugs are not only extremely expensive, but extend life by months, rather than years.

But now, much of the thrust of cancer research is based on an entirely different approach. Rather than treating leukemias with known leukemia drugs, or melanoma with melanoma drugs, a much more sophisticated approach is now the cutting edge of technology determining the mutation of genes that are responsible for cancers (and other diseases), and targeting the mutation not the broad cancer type.

The latest breakthrough in this area is a drug called venetoclax, which is being co-developed by Roche and AbbVie. Venetoclax specifically targets relapsed or refractory (non responsive) chronic lymphocytic leukemia (CLL) a cancer with a poor outcome that is caused by a specific genetic mutation on one chromosome.

Although only three to ten percent of CLL patients have this mutation, this number jumps to between 30 and 50 percent for patients who do not respond to conventional treatment. The bottom line: patients with the worst type of CLL benefit the most.

The FDA has (rightly so) designated venetoclax as a breakthrough therapy. This designation is given when early data show that a product may provide substantial improvement over existing therapies, especially in the case of life-threatening diseases. This designation gives a drug fast-track status with the agency, which allows it to jump ahead of other drugs and be reviewed more quickly.

Sandra Horning, the Chief Medical Officer and Head of Global Clinical Development at Roche said, We are pleased that the FDA has granted venetoclax breakthrough therapy designation and hope this regulatory pathway will help us bring venetoclax to people with this difficult-to-treat disease soon.

Dr. Bloom says, This is hardly the first time that treatments for diseases are based on gene sequencing. Although this is a major focus in oncology, the approach has been used in other disease areas. For example, Boston-based Vertex designed a drug called Kalydeco [3], which is used for treatment for a small set of patients who have a form of cystic fibrosis that arises from a specific genetic mutation. Only *four percent* of CF victims have this particular mutation, and the drug works only on this subset of patients. (This is also the perfect example of an orphan drug.)

This is just another example of the infancy of personalized medicine, which we can only hope will be the wave of the future.

Dr. Bloom predicts, I suspect there will be a time, perhaps in the near future, where traditional chemotherapy will be viewed as the modern equivalent of bloodletting. Then, the term cancer will take on an entirely different meaning, possibly in the same way HIV was 20 years ago.

Links

[1] https://www.centerwatch.com/drug-information/fda-approved-drugs/therapeutic-area/12/oncology
[2] http://nypost.com/2013/05/03/searching-for-the-wrong-miracles/
[3] http://www.kalydeco.com/