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Dr. Marlene Haffner

MD, MPH, (retired Rear Admiral in the USPHS)

Dr. Haffner's heads Haffner Associates and works with organizations focusing on rare diseases to help them form constructive partnerships with researchers and pharmaceutical companies to create and approve drugs for their condition.

For 20+ years Dr. Haffner, a now-retired Admiral in the Public Health Service Commissioned Corps, was the Director of the Office of Orphan Products Development at the Food and Drug Administration (FDA). During her tenure in that program, it became the outstanding orphan products program in the world.

Subject Area/Topic: Orphan Drugs

Highlights: Publication in 1979 of the Task Force report, "Significant Drugs of Limited Commercial Value", was a key step in focusing attention on the need for orphan drug development. It was followed by the enactment of the Orphan Drug Act of 1983, which was designed to facilitate the development of drugs for the treatment of diseases that apply to 200,000 or fewer individuals in the USA. Among other things, the Act authorizes 7-year market exclusivity, an FDA filing fee waiver, tax credits for clinical trials, orphan product grants and an accelerated approval process.

- Thirty million people in the US are living with a rare disease (one that applies to 200,000 people or less).
- 95% of rare diseases have no FDA approved drug treatment. (There are over 7,000 rare diseases and approximately 500 drugs to treat them.)
- 80% of rare diseases are genetic in origin.
- Approximately 50% of those affected by rare diseases are children.
- 30% of children with rare diseases will not live to see their fifth birthday.
- 8 is the average number of physician visits before there is a correct diagnosis.
- 3 is the average number of misdiagnoses.
- 7+ years is the average time before the correct diagnosis is made.

Dr. Haffner sees a very positive future for orphan drugs. The estimated worth of the global market is \$50 billion. 29% of orphan drugs have annual sales of over \$1 billion. There are indications that 15% of orphan drugs can be used to treat other rare diseases. And, as of 2013, 452 orphan products were under FDA review.