

Spotlight Interview on ducking the “Doughnut Hole”: the U.S. Rare Disease Health Cover Dilemma

Interview with Maria Hardin – Vice President, Patient Services
NORD, the US National Organisation for Rare Diseases

OrphaNews: Can you briefly explain the social and political background which has created the difficult financial situation in which many RD patients now find themselves?

There are many reasons why over 48 million Americans, including many individuals dealing with rare diseases, find themselves without any health insurance. During the earlier part of the 20th century, most major employers provided their employees with health benefits. Since then, over the past 20 years, skyrocketing costs for insurance premiums have caused many employers to drop health care benefits, or are forcing employees to pay a larger share of the costs. Workers on the low end of the socio-economic spectrum (many of whom earn only a minimum wage) are either not offered any benefits at all by their employers, or if they are offered them, find their share of the premium costs is prohibitively expensive, and choose not to join a health plan.

The US Government (both federal and state) is cutting back in health service provisions even to the lowest economic level citizens. In the States, insurance companies can pick and choose whom they will insure. People with chronic, life-debilitating conditions (many of these are rare diseases) find themselves classified as “uninsurable” even if they manage to pay the high premiums. Some states have established “high risk” insurance pools for their citizens but costs are high, and there is usually a 6-12 month waiting period for “pre-existing” conditions to be covered.

Tell us something about Medicare and its administration in the States

Medicare was established back in the mid 1960’s during the Lyndon B. Johnson administration. Both elderly (65 years and older) as well as younger, permanently disabled citizens were automatically enrolled in Part A that covers hospital costs. Part B covers medical services but requires payment of a nominal premium, and is optional. Prior to the passage of Medicare Part D, if citizens wanted coverage for their prescription drugs, they could purchase “Medi-Gap” insurance policies from the private insurance industry that were sanctioned by the U.S. Centers for Medicare and Medicaid Services, the government oversight agency for Medicare. The U.S. Congress passed the Medicare Modernization Act in late 2003, and for the first time, a prescription drug benefit was finally offered beginning January 1, 2006.

What exactly is this “doughnut hole” everyone seems to be talking about your side of the Atlantic?

It is the “share-of-cost” incorporated into the new Medicare prescription drug benefit program to off-set the total cost of the drugs to the government’s budget. Unfortunately, it is positioned at the “front end” of the beneficiary’s coverage year. The beneficiary pays the first \$250 for his/her Rx (called a deductible); then s/he must pay 25% of drug’s cost, and the insurance plan pays the other 75% until a total of \$2,250 has been reached. After that occurs, the beneficiary must pay 100% of the Rx cost until another \$2,850 is spent. This is called THE DOUGHNUT HOLE! If one adds \$2,250 + \$2,850 = \$5,100 at this point, the catastrophic level of coverage has been reached, and the beneficiary pays 5% per month for needed drugs for the remainder of the calendar year. Then the cycle begins all over again. In 2007, the out-of-pocket cost to the beneficiary extends to \$3,850.

If a beneficiary can prove that his/her annual income is less than a specific dollar figure, special help is provided by the government and there is no deductible, no doughnut hole, and only a

minimal co-payment for each prescription filled. Of the 13.2 million beneficiaries eligible for low-income assistance, 9.5 million are receiving the subsidy. However, there is an estimated 3.1 million eligible beneficiaries that are not receiving assistance.

NORD will be 24 years old next year. Your organization has been running Medication Assistance Programmes since starting up in 1987. How do these programmes help patients and their families cope with funding their rare disease treatments?

Thousands of uninsured patients have benefited from these programs. What NORD attempts to do is convince the pharmaceutical manufacturer that while the drug is still in the clinical trial stages, they need to incorporate a free drug assistance program into the company's launch plans so that, once the drug is FDA-approved and ready to be available commercially, access to the drug will be available even for those who do not have health insurance coverage. This also requires keeping the patient organizations apprised during the pre-launch period so that all avenues of access are widely known.

For patients who DO have prescription benefits either through Medicare or through regular private health insurance, but cannot afford their share of costs, we try to convince the manufacturers to make financial donations in support of a particular disease-state fund (ex: Multiple Sclerosis), not through a drug-specific fund. The challenge is to get all manufacturers of drugs for a given disease state to contribute, because for this to work, all the companies must contribute ...a very difficult task for NORD, since each company feels they may be aiding their competitors. Strict government regulations that deal with fraud, abuse, and steering issues have been established and do not allow manufacturers to pay beneficiaries directly for their out-of-pocket costs in order to access a particular drug, so charities like NORD play an important role in serving as the "go-between" in helping these individuals.

Of the estimated 50 million people in the US currently concerned by lack of funds for medical treatment, does NORD have an idea of how many are affected by RD's ?

Based on NIH estimates that between nine and ten percent of the US population is affected by a rare disease, one can extrapolate from the most recent figures recorded (July 1, 2005) from the U.S. Census Bureau that out of a total U.S. population of 296,410,404, that 28,158,988 suffer with a rare disorder*. FDA-approved therapies are usually expensive, and not always available on the insurance plans' formularies (ie the lists of drugs they will cover). If the rare disease afflicts the person to the extent that they cannot work, they may not have any health insurance. We believe there could be a significant number of that 50 million figure who are dealing with a rare condition.

**** NB: rare diseases in the US are determined by different criteria to those used in Europe. Instead of a maximum prevalence of 5 / 10,000, the limit used in the US is 7.5 / 10,000.***

On what level do these "free drug programmes" operate? How are they funded?

Just about every U.S. pharmaceutical manufacturer has established a free drug programme. Some are administered "in house" by the manufacturer, and others are contracted out to charities like NORD, or to a "for-profit" business that deals in screening patients for eligibility. The allotment of free drugs earmarked for such a program is usually based on a percentage of the commercial sales figures that could run from 1% to 10%. The lower the allotment provided to the program, the tighter the eligibility requirements (both financial and medical). The manufacturers pay an administrative fee to the entity that is screening the patients. This is usually based on the number of patients applying for assistance. There is usually a dispensing agency (mail order pharmacy) contracted by the manufacturer to ship the drug directly to the patient's home, or to the prescribing physician's office.

What does NORD think of the future of RD drug programmes in the US? Predictions?

We are disappointed that the new Medicare program seems to be gravitating towards becoming completely “privatized” by the present administration. They believe that only the private insurance companies can negotiate effectively for the lowest prices with the pharmaceutical manufacturers. The Medicare Modernization Act states that the government cannot negotiate for lower drug prices. A Medicare beneficiary in need of a very expensive drug (determined to be over \$500 per prescription) usually finds that the needed drug has been automatically placed in an insurance plan’s “specialty tier” that requires a higher co-payment, and one cannot appeal its placement to a lower, less expensive tier. NORD believes this was not the intent of the Act as written by the U.S. legislative body,

It is possible to identify political influences with regard to this issue, in the lead-up to the next Presidential elections and beyond?

The U.S. government has two main political parties: Republicans and Democrats. We have recently completed mid-term elections in a tight battle that put the Democrats back in control of the House of Representatives and on an even level in the Senate...something that hasn’t occurred in almost 12 years. Campaign promises made by Democrats running for office this year addressed the issues mentioned above, so it remains to be seen what the outcome will be for any changes to the Act.