Latest News and Updates from the Personalized Medicine Coalition

Letter from the Executive Director

In a statement issued on the 5th February 2009 at a congressional hearing on patient care, Massachusetts Senator Edward M Kennedy noted, "Less than half of all medical care in the nation is supported by adequate evidence of its effectiveness." Because nearly US\$2 trillion is spent annually on healthcare in the USA, or more than 15% of the nation's gross domestic product, it is widely believed that the USA, as well as other developed countries, can reduce the cost of healthcare by gaining a better understanding of how the money is spent and redirecting it more efficiently.

This contention has given rise to a new assumption that 'comparative effectiveness research' can introduce efficiencies into a system that is allegedly spinning out of control. It postulates that evidence-based knowledge derived from clinical trials that compare, say, two similar treatments, can determine what works best and thereby save money by eliminating usage of less effective, presumably more costly, approaches. Significantly, President Obama's fiscal stimulus package includes over US\$1 billion divided among the Agency for Healthcare Research and Quality (MD, USA), the National Institutes of Health (MD, USA) and the Secretary of the US Department of Health and Human Services (DC, USA) for such research.

The Personalized Medicine Coalition ([PMC], DC, USA) strongly supports efforts to expand the evidence base for healthcare decision making. It recognizes that expanding access and controlling spending must be part of any meaningful healthcare reform. But the PMC is equally committed to preserving the essential principle that policymakers should follow the science, not force providers and patients to make choices that more often than not are based on yesterday's data. They should seek to ensure that efforts to control costs do not harm patients who would be denied therapies that would work better for them, or stymie medical advances that could be derived from new scientific insights.

The PMC contends that comparative effectiveness research cannot be less sophisticated than the increasingly available molecular-based understanding of the etiology and progression of disease that has given rise to new therapies to treat many forms of cancer. For example, acute lymphoblastic leukemia, the most common form of cancer among children and adolescents, was a virtual death sentence a generation ago. Today, with the use of genetic screening and variation in treatment based on a patient's particular needs, survival rates are approaching 90%. If we do not incorporate our understanding of individual variation at the molecular level, comparative effectiveness could lock us into an obsolete, one-size-fits-all paradigm based on a simplistic, population-based law of averages that will not improve quality, foster medical progress or even reduce costs. In short, comparative effectiveness research that fails to consider today's biomedical breakthroughs will do more harm than good.

No matter how compelling they seem in the short term, policymakers and regulators therefore should eschew proposals that:

- Seek to contain costs by promoting the least expensive treatment on average, rather than the best care for the individual;
- Impose access restrictions based on average, population-wide study results;
- Discourage physicians from using gene-based tests and targeted therapies to optimize care for the individual;
- Ignore patient preferences based on their unique situations.

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Paradoxically, the principles of personalized medicine, properly employed, can lead to both improved clinical results and reduced costs. For example, a new study has found that US\$600 million could be saved annually if the anti-EGFR therapies panitumumab (Vectibix®) or cetuximab (Eribix®) were limited to those patients with metastatic colorectal cancer whose *KRAS* gene is not mutated, because those are the only patients who benefit from the drugs. Amgen (CA, USA) and Bristol-Myers Squibb (NY, USA), the companies marketing Vectibix and Eribix, are seeking US FDA label changes to recommend or require the diagnostic test, which would lead to better care and cost savings by eliminating unnecessary prescriptions.

Comparative effectiveness research that incorporates an appreciation of newly discovered methodologies in genetics and genomics, genome-wide association studies and the role of genes in drug metabolism, to name a few recently developed scientific strategies, has enormous potential to improve patient care and advance intelligent healthcare reform. If it is done poorly and fails to recognize breakthroughs in medical science, it will neither help patients nor repair broken healthcare systems.

**Edward Abrahams, Executive Director,
 Personalized Medicine Coalition**