As far as we know, Hippocrates was the first to formally recognize nutrition as an absolute therapeutic necessity. He is credited with memorable, and still remarkably relevant, quotes such as, “Let food be thy medicine and medicine be thy food,” and “Leave your drugs in the chemist’s pot if you can heal the patient with food.”

More than 2 millennia later, the goal of healthcare—getting the right therapy to the right patient at the right time—hasn’t changed all that much; however, our collective approach to incorporating new technologies in the treatment armamentarium must evolve. The following anecdote is a real-world example of what is happening in “niche” technology development across our industry.

**An Age-Old Health Issue**

I’ll begin with the very basic premise that adequate nutrition is vital to physiologic functioning and psychological well-being. For patients with a broad range of medical conditions, inadequate nutrition and/or ineffective nutritional support further compromise their health status and increase the cost of their care.

The term “malnutrition” may call to mind images of children and adults living in underdeveloped and developing countries. It may come as a surprise to many Americans, but physicians regularly diagnose and treat patients with malnutrition in intensive care units (ICUs) and in skilled nursing facilities across the United States. For the most medically advanced country in the world, this is unacceptable, even on a relatively small scale.

**Target Populations**

A variety of conditions are associated with digestive deficiency or malabsorption, such as patients who rely on tube feeding for nutrition, patients in ICUs, and infants in neonatal ICUs.

As a result of a lack of adequate pancreatic digestive enzymes, patients with exocrine pancreatic insufficiency have clinical symptoms related to malabsorption of fat. Exocrine pancreatic insufficiency is associated with diseases and conditions that affect the pancreas, including hereditary conditions (eg, cystic fibrosis) or acquired conditions (eg, chronic pancreatitis). For many of these patients, enteral nutrition is necessary to avoid malnourishment. Particularly in patients with cystic fibrosis, good nutritional status has been associated with improved clinical outcomes.

**Limited Therapeutic Options**

None of the current US Food and Drug Administration (FDA)-approved pancreatic enzyme replacement therapies (PERTs) are indicated for use in patients who receive enteral nutrition for several reasons. PERT capsules are intended for oral use; when crushed and added to enteral nutrition feedings, they have been shown to clog the tubes. In addition, the capsules are not indicated for overnight enteral nutrition, because their activity peaks at 30 minutes and wanes thereafter. Most important, even when administered in large doses, PERT capsules do not resolve the issue of incomplete hydrolysis of fats from enteral nutrition. Many patients with exocrine pancreatic insufficiency who use PERT capsules continue to struggle nutritionally and have clinical symptoms related to fat malabsorption.

**Innovative Solution**

A new hybrid technology was developed to address the treatment gap for the population of patients with conditions associated with digestive deficiency and malabsorption, in particular for patients who rely on tube feeding for nutrition, patients with acute pancreatitis and/or pancreatic cancer, children with cystic fibrosis, patients in ICUs, and infants in neonatal ICUs.

Immobilized lipase (Relizorb) is a novel in-line digestive cartridge designed specifically for hydrolyzing fats in enteral formulas. Administered without the risk for clogging feeding tubes, this technology has been shown to break down more than 90% of fats in enteral nutrition formulas throughout feedings.

This hybrid technology was cleared by the FDA in December 2015 after a lengthy approval process. There is growing real-world clinical evidence of tolerability and improved fatty acid absorption, including weight gain and increased patient adherence to oral nutrition in patients with cystic fibrosis. Specifically, cystic fibrosis centers that use this technology to treat patients with
cystic fibrosis who are receiving enteral nutrition have documented weight gain and increased basal metabolic rate. The patients included in short- and long-term studies of the technology at these centers represent roughly 2% of the approximately 3600 patients with cystic fibrosis who are being fed via a feeding tube.5-8

The Value Question
I’ve heard it said that technology generates value only if the health benefit outweighs the cost. Herein lies the next hurdle—getting a firm grasp on costs. In the example of this new treatment, the direct costs associated with enteral nutrition can range from $80 to $200 daily, considering the cost of the formula, tube-feeding supplies, and oral pancreatic enzymes (ie, PERT). Despite its significant expense, patients with cystic fibrosis who receive enteral nutrition achieve only 60% of normal fat levels, even after years of treatment.4 As in patients with other conditions related to exocrine pancreatic insufficiency, hospitalization becomes necessary when malabsorption results in malnutrition. The in-hospital costs of enteral nutrition include the time spent by medical professionals crushing and adding PERTs to feeding bags and addressing unresolved symptoms in patients.

Clearly, the potential benefits of innovative therapies for their target populations are substantial, and can be life-changing for some people. But how do we weigh cost versus health benefits when the target population is extremely rare? In the United States, for example, only approximately 11% of patients with cystic fibrosis (approximately 3300 individuals) required supplemental enteral nutrition in 2014.9 Similar to many innovative treatments, this new technology does not fit the typical payer reimbursement model.

Payer Implications
Payers consistently reimburse providers for standard therapies, even those that often fail to achieve the intended goal, such as traditional enteral nutrition regimens. In this case, the use of a new technology may optimize enteral nutrition, enable patients to achieve nutritional goals, reduce or eliminate the need for PERT during tube feeding, and ultimately reduce the avoidable costs associated with frequent hospitalizations and increased length of stay.

My takeaway message is that targeted innovative therapy is the new reality, one that has unimaginable promise for subpopulations of patients who struggle with relatively rare conditions. All stakeholders must accept responsibility for cutting through the politics to clear the pathway and get the right therapies or new technologies to the right people at the earliest opportunity.

Disclosure Statement
The general concepts and questions addressed in this editorial come across my desk with growing frequency. The issues are complex and a resolution will require multistakeholder input. To that end, we are working with the developers of this new technology to convene a national advisory panel.

References