

Below are responses from the Cystic Fibrosis Foundation to questions issued by the Institute of Medicine (IOM) on the Determination of Essential Health Benefits, a component of the Patient Protection and Affordable Care Act. The CF Foundation responded to questions relevant to the needs of people with cystic fibrosis (CF), highlighting the need for specialized, comprehensive care and access to CF specialists in accredited care centers. These responses were submitted to the IOM on December 6, 2010.

CFF Comments to the Institute of Medicine regarding the Determination of Essential Health Benefits

IOM link: <http://www.iom.edu/Activities/HealthServices/EssentialHealthBenefits.aspx>.

Question 3

What is your interpretation of the word “essential” in the context of an essential benefit package?

The term “essential” must be interpreted to include the full scope of services and goods necessary for delivery of quality health care. For individuals with cystic fibrosis (CF), that standard requires daily access to multi-disciplinary care without cost-sharing requirements that limit access. For the individuals with serious and chronic illnesses, maintaining good health requires adherence to a complex daily regimen of care. Those patients will be unable to maintain good health if cost-sharing responsibilities cause them to forgo treatment, reduce medication dosages, or substitute less effective medications for recommended medications.

The definition of “essential” must also permit the rapid incorporation of innovations into the benefit package. The improved care and survival of those with CF has depended on a rich pipeline of new therapies, but those treatments will only make a difference if the benefit package facilitates their immediate incorporation into routine care.

Question 4

How is medical necessity defined and then applied by insurers in coverage determinations? What are the advantages/disadvantages of current definitions and approaches?

The definition of medical necessity, which relies on the concept of a good or service being “reasonable and necessary,” is applied with inadequate flexibility to cystic fibrosis care. It is important that the definition of medical necessity include deference to the physician and patient to make decisions about care, consistent with standards of care that are developed and defined by the CF research and clinical care experts on the basis of the best available evidence. Consistent with the defined standards of care, patients and their caregivers must have the ability to refine and personalize the treatment regimen to meet their specific needs.

The application of the “reasonable and necessary” standard also generally excludes from coverage care like nutritional supplements and certain other elements of care that are critical for those with CF. A more flexible application of the standard to include items and services not

traditionally considered “health care” would help those with CF and other chronic diseases maintain good health and would prove a wise investment for insurers through prevention of serious complications of CF.

Question 5

What criteria and methods, besides medical necessity, are currently used by insurers to determine which benefits will be covered? What are the advantages/disadvantages of these current criteria and methods?

Insurers are increasingly analyzing cost of therapies in making decisions about coverage of treatments, to the significant detriment of patients. For example, there is insufficient flexibility in the coverage of pancreatic enzymes, with coverage decisions often driven by cost. These decisions limit the access of patients to the enzymes that work most effectively for them. Cost also appears to influence the pace of decisions about coverage, translating into a reluctance to adopt and embrace new technology and new treatments.

The analysis that seems to be applied does not reflect a long-term view, which would demonstrate the potential savings from coverage of drugs and other therapies that are effective on an individual basis and that can help CF patients remain healthy and prevent infections and pulmonary exacerbations.

We are aware of the strong and ongoing federal investment in comparative effectiveness research, and we appreciate that there will be an important national debate regarding the appropriate use of comparative effectiveness research results to inform coverage and payment policies. We are referencing not the potential for comparative effectiveness research to provide data that would be considered as part of coverage analyses. Instead, we refer specifically to cost-driven decisions to deny access to safe and effective therapies that may have potential to extend lives and to improve quality of life.

Question 6

What principles, criteria, and process(es) might the Secretary of HHS use to determine whether the details of each benefit package offered will meet the requirements specified in the Affordable Care Act?

We recommend that the Secretary utilize the clinical guidelines and practice guidelines developed by medical and clinical care organizations and societies to determine the adherence of benefit packages to the Affordable Care Act requirements. In the case of CF, standards of care are well-defined by the experts who daily treat individuals with CF and who are engaged in CF clinical research. These standards are the most effective measure of the benefit package.

It is critically important that the Secretary also implement a process that is timely and efficient, so that changes and improvements in the standard of care are noted and utilized in determining the adequacy and appropriateness of each benefit package.

Question 7

What type of limits on specific of total benefits, if any, could be allowable in packages given statutory restrictions on lifetime and annual benefit limits? What principles and criteria could/should be applied to assess the advantages and disadvantages of proposed limits?

The Cystic Fibrosis Foundation strongly opposes the application of limits on any specific benefit class or category or any category of drugs. Such limits, which are currently applied by many insurers, have a serious adverse impact on those with CF, who often require products or services on a more frequent basis or on a more intensive basis than the general population. Because those with CF are essentially outliers in the intensity of services they require, limits on any class of benefits have a disproportionate impact on them.

Moreover, limits on any class or category of good or service are inconsistent with the intent of Congress in restricting the application of lifetime and annual spending limits. Limits on spending by class of benefit have the potential to limit access to care for those with chronic disease, with adverse health care outcomes a likely result.

Question 8

How could an “appropriate balance” among the ten categories of essential care be determined to that benefit packages are not unduly weighted to certain categories? The ten categories are: ambulatory patient services; emergency services; hospitalization; maternity and newborn care; mental health and substance use disorders services, including behavioral health treatment; prescription drugs; rehabilitative and habilitative services and devices; laboratory services; preventive and wellness services and chronic disease management; pediatric services, including oral and vision care.

The Cystic Fibrosis Foundation believes that an appropriate balance will be achieved among the ten categories of essential care if those services are covered and reimbursed according to the clinical guidelines developed by medical and professional societies. Individuals with CF may require services for each category or care over the course of a year and almost certainly will require the full scope of services over a lifetime. The balance among the categories can be achieved on an individual basis, and ultimately on a plan or population basis, if care is provided according to the best available evidence.

CF patients and their parents play a critical role with their care teams in coordinating their care. They do this of necessity, but the planning and coordination of care will also help ensure the proper balance among the categories of care. By proper planning and coordination, there can be some assurance that expensive emergency care will not be over-utilized, for example.

Question 9

How could it be determined that essential benefits are “not subject to denial to individuals against their wishes” on the basis of age, expected length of life, present or predicted disability, degree of medical dependency or quality of life? Are there other factors that could be determined?

For many individuals with CF, denials of care result from the unmanageable financial burden of care. In the past, a disturbing minority of those with CF triggered annual or lifetime insurance spending limits, and many more were effectively denied care because they could not meet cost-sharing requirements. Although the Affordable Care Act will address issues related to lifetime and annual spending limits, it will not entirely address the issue of significant cost-sharing. Because of their intensive utilization of health care services, those with CF may not be able to shoulder the cost-sharing associated with their care and may alter their plan of care based on their ability to pay.

The Secretary can address this issue by seeking not only a balance among the categories of essential benefits but also by requiring reasonable cost-sharing requirements for all categories and special attention to the burden of cost-sharing on those with chronic diseases.

Question 10

How could it be determined that the essential health benefits take into account the health care needs of diverse segments of the population, including women, children, persons with disabilities, and other groups?

The Secretary must develop a system for rapid feedback about the essential health benefits package and its ability to address the health care needs of diverse populations.

The population of those with CF is small in number, but even within this orphan population there are diverse health care needs. Those with CF experience varying degrees of severity of the disease, and the needs of children are different from those of young adults and older adults. The only way to ensure that the essential benefits package meets the diverse needs of

this small population is to establish a mechanism for rapid and substantive feedback from medical professionals and patients regarding the adequacy of the essential benefits.

Question 11

By what criteria and method(s) should the Secretary evaluate state mandates for inclusion in a national essential benefit package? What are the cost and coverage implications of including current state mandates in requirements for a national essential benefit package?

We recommend that the Secretary evaluate the state coverage mandates on the basis of benefit to consumers and cost to consumers and insurers. By this measure, we anticipate that there will be a strong case for including some of the current mandates in the benefit package and a less compelling case for others.

We believe the current mandates for coverage of routine patient care costs in clinical trials will be evaluated positively, as states have found the cost of clinical trials coverage to be minimal while patients and physicians have reported positive results from eliminating the economic barrier to trial participation. We encourage the Secretary to set an essential benefits package standard for clinical trial coverage for all diseases, a modest modification to the Affordable Care Act provision for coverage of cancer clinical trials.

Question 12

What criteria and method(s) should HHS use in updating the essential package? How should these criteria be applied? How might these criteria and method(s) be tailored to assess whether: (1) enrollees are facing difficulty in assessing needed services for reasons of cost or coverage, (2) advances in medical evidence or scientific advancement are being covered, (3) changes in public priorities identified through public input and/or policy changes at the state or national level?

Cystic Fibrosis Foundation recommends that the Secretary establish a policy that would require the incorporation of products approved by the Food and Drug Administration into the benefit package. A clear standard for inclusion of new technology in the benefit package is important for those with CF and other chronic diseases, who depend on new medical developments to extend their lives and enhance their quality of life.

In addition, we urge that the Secretary develop a means for updating the essential benefit package, consistent with advances in medical evidence or clinical practice. We propose that the process for updating the package rely on the recommendations of the medical experts, in scientific or medical societies, regarding advances in care for specific populations of patients.