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CONTACT: Siri Vaeth
Email: svaeth@cfri.org

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CYSTIC FIBROSIS RESEARCH INC. STATEMENT ON ICER'S CYSTIC FIBROSIS EVIDENCE REPORT

Patient advocates extremely concerned about report's potential impact on access, interference with the physician-patient relationship

Palo Alto, Calif. – Cystic Fibrosis Research, Inc. (CFRI) is extremely concerned by the content of the Institute for Clinical and Economic Review's (ICER) draft evidence report on the value of cystic fibrosis treatments.

ICER's evidence report, released earlier today, evaluates studies measuring health outcomes for those using CFTR modulator therapies and compares their use to the cost of CF-related lung exacerbations and double lung transplants. Despite noting that these CFTR modulator therapies combined with best supportive care "substantially improve patient health outcomes compared to best supportive care," the report's authors in essence quantify the cost of a human life and conclude, "Because of the high cost of these drugs, however, the cost of CFTR modulator therapies exceed commonly used cost-effectiveness thresholds."

In direct contrast to these opinions, those who have personal experience with the disease, including cystic fibrosis clinicians, patients, family caregivers, and CF advocates, view these new treatments as life-changing because there are very few therapies approved to treat the underlying causes of this chronic condition. In clinical trials, these therapies have shown improved lung function, reduction in lung infections requiring hospitalization, and improvement in quality-of-life indicators.

The Institute for Clinical and Economic Review (ICER) purports to utilize a methodology to measure the effectiveness and cost-effectiveness of therapies to treat a variety of conditions. In addition to a review of relevant clinical trials, its methodology is said to include input from clinicians, advocacy organizations and individuals living with the disease. But members of the cystic fibrosis community - including numerous national cystic fibrosis organizations - have grave concerns that they were not sufficiently included in the process prior to the release of the draft report. While ICER has no medical nor governmental authority, there is significant concern that this report will be used by others to deny access to life-saving care.

Sue Landgraf, Executive Director of CFRI, noted that, "ICER's evidence report is exactly what we expected – it lays the predicate for public and private payers to deny cystic fibrosis patients access to these life-changing and groundbreaking therapies. The representation on ICER's governance board raises concern that this report is overly focused on cost savings. For individuals suffering with

cystic fibrosis, these new therapies can improve quality of life and reduce respiratory complications – a serious issue for these patients. We urge patient advocates and and clinicians to voice their concerns, as we at CFRI have done, directly to ICER so that access to these therapies is not jeopardized."

Public comment on the draft report will take place May 17th 2018, at the Midwest Comparative Effectiveness Public Advisory Council in St. Louis MO. CFRI's Associate Director, Siri Vaeth, will be there to express CFRI's significant concern with the draft report. Says Vaeth, "This report is focused on number crunching, and completely removes the human cost of the disease from the evaluation process. Our community is suffering. Last year the median ago of death for those with CF was only 30 years – usually after a long and debilitating health decline. This report does not reflect the interests of the cystic fibrosis community, and may, in fact, prevent those with CF from receiving needed care."

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About CFRI: Cystic Fibrosis Research, Incorporated funds research, provides educational and personal support, and spreads awareness of cystic fibrosis, a life-threatening genetic disease. CFRI seeks to inform, engage and empower the CF community to reach the highest possible quality of life, as we work towards a cure.