

July 1, 2004

Department of Health and Human Services
Division of Dockets Management
U.S. Food and Drug Administration

Re: Electronic Submission for Docket No. 2004S-0170—Medicare Prescription Drug, Improvement, and Modernization Act of 2003, Section 1013: Priority Topics for Research

Dear Sir or Madam:

The Juvenile Diabetes Research Foundation International (JDRF), the American Association of Clinical Endocrinologists (AACE), and Medtronic appreciate the opportunity to submit recommendations for topics of research to be conducted under Section 1013 of the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA). Section 1013 of the MMA authorizes research, demonstrations, and evaluations to improve the quality, effectiveness, and efficiency of the Medicare, Medicaid, and State Children's Health Insurance Program (SCHIP) entitlement programs.

JDRF, AACE, and Medtronic are dedicated to improving the quality of care for individuals with juvenile (Type 1 insulin-dependent) diabetes. Since 1970, JDRF has been a leading charitable funder and advocate of Type 1 diabetes research. JDRF sponsors innovative, cutting-edge research worldwide and is focused on accelerating research progress to cure diabetes and its complications. Founded in 1991, AACE represents over 4,800 clinical endocrinologists worldwide and remains committed to transforming the lives of patients by promoting the practice of leading edge, proactive, ethical, and cost effective medicine. Medtronic is the world's leading medical technology company and a world leader in insulin pump therapy and continuous glucose monitoring systems.

Our research recommendations focus on the quality of care for Type 1 diabetes. Despite the widespread literature conclusively indicating the benefits of intensive insulin management, many patients with Type 1 diabetes are not being intensively managed and are not meeting established treatment targets. Understanding the challenges of delivering quality diabetes care, including intensive insulin management, to individuals with Type 1 diabetes in Medicare and Medicaid programs should be a primary research objective for the Agency for Healthcare Research and Quality's (AHRQ's) fiscal year (FY) 2006 research priorities under Section 1013 of the MMA.

Below, we provide background information on Section 1013 of the MMA, discuss the importance of Type 1 diabetes in terms of the public programs of interest, and provide more detailed information regarding our research recommendations.

Background

As indicated in the MMA, research and other activities undertaken and authorized by Section 1013 may address: (1) The outcomes, comparative clinical effectiveness, and appropriateness of health care items and services (including prescription drugs); and (2) Strategies for improving

the efficiency and effectiveness of Medicare, Medicaid, and SCHIP programs, including the ways in which health care items and services are organized, managed, and delivered under such programs.

For its role in implementing Section 1013, AHRQ has requested public input for suggestions regarding which technologies and conditions should be included in their research priorities for FY 2006. JDRF, AACE, and Medtronic applaud AHRQ's efforts in requesting public input for research topics. Recent discussions indicate that for the first year of funding, investigations into the effectiveness of drugs and emphasis on conditions that most impact the health of beneficiaries should be an initial priority. Additional topics to be considered for the FY 2006 priority list include suggestions that may address a broader range of other health care items or services that focus on outcomes and the quality of patient care.

Prevalence and Cost of Type 1 Diabetes in Public Programs

One area that meets the criteria as an opportunity for improved quality and cost savings in public programs is the treatment of Type 1 diabetes. Individuals with Type 1 diabetes are an easily identifiable and treatable patient population. In addition, the cost-savings associated with the proper management of individuals with Type 1 diabetes have been well documented. According to the American Diabetes Association (ADA), 1 million people currently are diagnosed with Type 1 diabetes and 30,000 new cases of Type 1 diabetes are diagnosed annually. The onset of Type 1 diabetes can occur at any age, but it is one of the most frequent chronic diseases among children in the United States. Overall, over half of individuals with Type 1 and 2 diabetes are covered by government-financed health insurance programs.¹ This includes children and low-income adults covered under Medicaid and SCHIP, as well as the elderly, disabled, and individuals with end-stage renal disease (ESRD) covered by Medicare.

Type 1 diabetes is a chronic illness and a disproportionately expensive disease. According to a 2002 study published in *Diabetes Care* and endorsed by the National Institutes of Health and Centers for Disease Control and Prevention, direct medical and indirect expenditures attributable to both Type 1 and Type 2 diabetes in 2002 were estimated at \$132 billion. Although individuals diagnosed with diabetes accounted for only 4% of the U.S. population in 2002, health care costs associated with diabetes represent roughly 19% of total personal health expenditures. Direct medical expenditures for diabetes totaled \$91.8 billion in 2002 with \$23.2 billion attributable to diabetes care and \$24.6 billion for chronic complications related to diabetes.²

Since publication of results from the landmark Diabetes Control and Complications Trial (DCCT) in 1993, many published studies have confirmed the importance of intensive diabetes self-management for individuals with Type 1 diabetes and the clinical benefits of lowering glycosylated hemoglobin levels (HbA1c), the generally accepted measure of diabetes control.^{3,4} DCCT defines intensive insulin management as three or more insulin injections per day or the use of a continuous subcutaneous insulin infusion device. Aside from lowering a patient's risk for serious health complications, reducing HbA1c levels and achieving recommended HbA1c targets also helps lower overall healthcare costs attributable to diabetes. Treatment guidelines established by medical organizations recommend that people with diabetes achieve an HbA1c that approaches near-normal levels (normal is 3.8-6 %) to reduce the risk for microvascular

complications that result in eye, kidney and nerve damage and macrovascular complications that result in severe cardiovascular events. Despite the widespread literature conclusively indicating the benefits of intensive insulin management, 10 years after the publication of DCCT, only 55% of patients with Type 1 diabetes in the U.S. were being intensively managed in 2003.⁵

Medicare and Diabetes

Diabetes is associated with increased mortality in the 65 and older population and improved quality of care for Medicare beneficiaries with diabetes has been outlined as a recommended public health goal.⁶ According to 2002 data from the AHRQ Medical Expenditure Panel Survey (MEPS), Type 1 and 2 diabetes accounted for \$5.2 billion in total Medicare expenditures, making it the ninth most costly condition for the Medicare program. Data from JDRF indicate that diabetes and diabetes-related complications represent 25% of all Medicare expenditures.⁷ Recent literature also indicates that Medicare beneficiaries, especially dual-eligibles covered both by Medicare and Medicaid, are less likely to receive diabetes care and, therefore, have higher rates of diabetes complications and associated costs.⁸

The ADA denotes diabetes as the leading cause of ESRD, accounting for 43% of new ESRD cases. In 2000, 41,046 people with diabetes began treatment for ESRD and in 2000, 129,183 individuals with diabetes underwent dialysis or kidney transplantation.⁹ According to data from the Centers for Medicare and Medicaid Services (CMS), diabetes is the primary cause of ESRD for one-third of all Medicare ESRD beneficiaries. Patients with ESRD, particularly patients on dialysis, are one of the most costly populations for the Medicare program and have high morbidity and mortality rates. Early intervention related to tight glycemic control and better management of chronic kidney disease and its underlying causes, such as diabetes, may delay or even prevent permanent kidney failure and ESRD. Medicare spent over \$14 billion for ESRD patients in 2001, demonstrating that the cost of diabetes to the Medicare program goes beyond simply treatment of the disease itself.¹⁰

Medicare has recently renewed its commitment to improve the quality of care of beneficiaries with diabetes as demonstrated by the following:

- ?? *Voluntary Chronic Care Improvement Program (CCIP)*: CCIP is aimed at improving the quality of care of people living with chronic conditions, including diabetes. CCIP will assist beneficiaries in managing their conditions and improve their coordination of care across health care settings and among service health care professionals. It also seeks to educate patients about how to care for themselves and promote the use of evidence-based treatment guidelines.
- ?? *Covered diabetes treatments*: Medicare currently covers a wide variety of diabetes-related services and treatments for its beneficiaries, including
 - blood glucose monitoring equipment and supplies,
 - diabetes self-management training,
 - medical nutrition therapy.

- insulin pumps, and the insulin used with the pumps, for certain beneficiaries. Currently, 8,000-9,000 Medicare beneficiaries are using an insulin pump.¹¹

?? *Preventative diabetes services:* Beginning in 2005, Medicare will cover diabetes screening tests for persons at risk for diabetes, including a fasting plasma glucose test. Also beginning in 2005, Medicare will cover a one-time preventive physical examination within 6 months of a beneficiary's enrollment under Medicare Part B. The exam will provide an opportunity to assess risk factors that could lead to complications of Type 1 diabetes.

?? *Prescription drug coverage and management:* In 2006, Medicare will begin to cover insulin and associated diabetes supplies, including syringes, under the Medicare Part D Prescription Drug Benefit. Additionally, the new prescription drug program will provide drug therapy management for beneficiaries with multiple chronic diseases (including diabetes) who take multiple drugs and who spend more than a specified amount of money annually on drugs covered under the prescription drug benefit.

Medicaid, SCHIP, and Diabetes

AHRQ MEPS data from 2000 indicate diabetes as the twelfth most costly disease for Medicaid with \$2.0 billion in expenditures for diabetes care and complications. Since state data regarding the number of Medicaid beneficiaries with diabetes are limited, it is unclear exactly how many patients with diabetes are eligible for Medicaid benefits, although data are available for certain states (Table 1). Furthermore, as of December 2003, 46 states had some type of laws requiring health insurance coverage to include treatment for diabetes (the states not included are Alabama, Idaho, North Dakota, and Ohio).¹²

Because Medicaid and SCHIP provides health benefits to the neediest patients, state Medicaid and SCHIP programs play an important role in terms of patient access to diabetes care. Moreover, although Medicaid and SCHIP patients with diabetes comprise a relatively small population, the costs associated with treating these patients and their diabetes-related complications are significant.

Table 1: Percentage of Population with Diabetes within Select States, 2001

State	Percent Diabetic Population in State ⁺⁺	Total Medicaid Population (thousands), by Category ⁺					
		TANF/ SSI recipients [*]	Medically needy	Poverty related	Other	1115 demo ^{**}	Unknown
CA	9 to 10%	3,973	860	158	1,299	1,943	0
CO	4 to 6%	211	0	152	47	0	<1
NJ	9 to 10%	419	5	331	169	0	0
TX	7 to 8%	910	124	1,197	494	5	0

⁺Source: Medicaid Statistical Information System (MSIS) Report for Federal Fiscal Year 2001.

<http://www.cms.gov/medicaid/msis/msis99sr.asp>.

⁺⁺Source: Mokdad, A. et al. (2003) Prevalence of obesity, diabetes, and obesity-related health risk factors, 2001. *Journal of the American Medical Association* 289(1):76-79.

^{*}TANF = Temporary Aid for Needy Families; SSI = Social Security Insurance.

^{**}The 1115 waiver program allows states to apply to the federal government for permission to expand Medicaid benefits in a cost-neutral way to additional populations not normally captured under the Medicaid program.

As state governments struggle to balance their budgets, Medicaid has become a frequent target of cost-cutting efforts. Patients and clinicians alike are concerned diabetes treatments may become the subject of budget cuts, thereby limiting access to care for the most needy diabetes patients. Such limitations are of particular concern for the more intensive insulin management services, which are more expensive and typically are targeted toward Type 1 diabetes. Moreover, denying supplies and services to individuals with diabetes in the short-term may lead to long-term conditions requiring expensive treatment and management and overall higher costs.^{13,14,15}

Research Recommendations

Further research on the challenges of delivering quality care, including intensive insulin management, to patients with Type 1 diabetes in Medicare and Medicaid programs should be a primary research objective in AHRQ's FY 2006 research priorities under Section 1013 of the MMA.

The high cost of diabetes in part underscores an unmet need for improved treatment of this patient population. Diabetes can be managed effectively to prevent many of the costs and long-term complications that currently are prevalent in the Medicare and Medicaid populations. Specifically, intensive insulin therapy has been shown to be cost-effective in both Type 1 and Type 2 diabetes because two-thirds of the costs of diabetes management currently are related to hospital inpatient care and associated complications.^{16,17}

Evidence continues to accumulate in support of the importance of a decrease in glycosylated hemoglobin (HbA1c) levels to 7.0% or lower if possible.^{18,19,20,21,22} DCCT found that patients who received intensive insulin therapy had significant reductions in the progression of diabetic complications compared to patients who received conventional insulin therapy. Most notably, patients who received intensive insulin management showed large reductions in nephropathy,

retinopathy, and neuropathy. These complications can result in substantial—and avoidable—costs to the health system.

Although AHRQ has funded research on diabetes care in the past, it has not focused on intensive insulin therapy and its impact on successfully managing Type 1 diabetes and related complications. Specific research questions that should be addressed include the following:

- ?? To what extent are patients with Type 1 diabetes achieving established intensive insulin management objectives?
- ?? How has the current body of knowledge for improving the health of individuals with Type 1 diabetes been translated into the development of clinical guidelines and adoption into clinical practice?
- ?? What are the potential barriers to the effective management of Type 1 diabetes including barriers at the patient and health care professional level and organizational and financial factors?

Each of these research questions is described in greater detail below.

Extent to Which Type 1 Diabetes Patients are Achieving Evidence-Based Targets

Despite the evidence regarding the benefits of intensive insulin management, as highlighted above, many patients with Type 1 diabetes do not achieve the recommended HbA1c targets of less than 7%. Based on our preliminary research, the average HbA1c of all patients with diabetes in the United States is 8.6% and up to one-third of diabetes patients have HbA1c levels above 9.5%.²³

Further information is needed to better understand exactly how many patients are not achieving clinical HbA1c targets and if there are sub-groups of patients with Type 1 diabetes that are disproportionately unlikely to meet these targets. For example, it is important to understand whether disparities exist in the outcome and treatment of patients in different public programs (i.e., Medicare, Medicaid, and SCHIP) and whether there are variations by ethnicity or geography.

Once it is better understood which patients with Type 1 diabetes currently are not being treated to clinically acceptable targets, additional research can be conducted to determine the likely reasons. A systematic review of existing literature would be an acceptable approach to address this question and to inform ongoing dialogue in this area.

Translating Research into Clinical Practice

Improving the way in which the healthcare community can translate research findings into clinical practice is an ongoing goal and challenge for AHRQ. We recommend conducting research regarding whether appropriate clinical guidance exists and reflects the current knowledge base for the treatment of Type 1 diabetes. We also recommend determining the extent to which available guidance is being used to treat Type 1 diabetes in clinical practice.

In terms of guidelines for Type 1 diabetes, the clinical community has identified specific quality indicators for the management of the disease, largely based on evidence from DCCT. The ADA guidelines recommend an HbA1c less than 7% and the American College of Endocrinology (ACE) and AACE recommend an HbA1c less than 6.5%.²⁴

Furthermore, organizations such as the National Committee for Quality Assurance (NCQA) have developed widely-used quality measures such as the Health Plan Employer Data and Information Set (HEDIS[®]) and the Diabetes Physician Recognition Program (DPRP) that include HbA1c control as a quality measure for adults and children with diabetes. The DPRP does not use the levels promoted in the AACE and ACE guidelines. Instead, the DPRP collects data on the percentage of children with HbA1c levels below 10% and 8% and the percentage of adults with HbA1c levels greater than 9% and less than 7%. The HEDIS[®] measures, which are widely used to evaluate health plan quality, focus on process (whether the patient received an HbA1c test in the last 12 months) and the percentage of patients who are in “poor” control, specifically those patients with HbA1c levels above 9.5%.²⁵

Further information is needed to better understand the level of clinical consensus to and knowledge of quality indicators for Type 1 diabetes and how sufficient existing clinical guidelines are in providing an actionable path for health professionals and patients with Type 1 diabetes. The quality of these guidelines and their adoption by the clinical community is crucial in moving research into practice and changing existing behaviors and policies.

Identifying and Examining Potential Barriers to the Effective Management of Type 1 Diabetes

Identification of barriers to achieve optimal control of diabetes at various levels of care management is crucial to improving the overall quality of care for Medicare, Medicaid, and SCHIP patients with Type 1 diabetes. We recommend a structured analysis to examine the patient-level, health care professional-level, and organizational and financial barriers to more effective management of Type 1 diabetes. Examples of barriers in each of these categories are as follows:

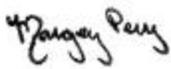
- ?? Patient-level barriers: Lack of patient awareness of and education about the importance of good HbA1c control; lack of access to quality care; and reluctance, unwillingness, or inability to comply with intensive therapy.
- ?? Health care professional-level barriers: Lack of health care professional awareness and education about successful diabetes treatment options including new insulins and delivery systems; lack of specialized training in the care of Type 1 diabetes; belief that patients will not be compliant with intensive management; concerns regarding hypoglycemia; shortage of specialists treating Type 1 diabetes (across the country or in specific geographic areas); and insufficient time and compensation to educate and monitor patients on intensive insulin management programs.
- ?? Organizational and financial barriers: Public payer policies that create disincentives for intensive management; insufficient reimbursement to use technologies that have proven benefit in the management of Type 1 diabetes; inadequate referral networks or

communication systems for specialty care for difficult-to-manage patients; and out-of-pocket costs to patients for intensive management.

Research should identify the barriers that play important roles in preventing better management of Type 1 diabetes and identify mechanisms to overcome these barriers, and solutions must be developed that are appropriate in the context of government-funded health care programs.

Focusing AHRQ's 2006 work under Section 1013 of the MMA on improving the quality of care delivered to individuals with Type I diabetes covered by public programs is an important first step toward providing such beneficiaries with the highest quality of care available. We thank the Department of Health and Human Services, FDA, and AHRQ for the opportunity to comment. If you have any questions regarding these recommendations, please contact Margery Perry, Chair of Research, JDRF at (800) 533-2873, or Carlos Hamilton, Jr, MD, FACE, President, AACE at (904) 353-7878, or Claudia Graham, PhD, MPH, Vice President, Global Therapy, Medtronic Diabetes at (818) 362-5958.

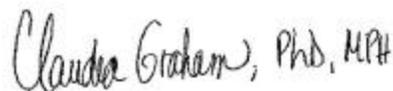
Sincerely,



Margery Perry
Chair of Research
JDRF



Carlos Hamilton, Jr, MD, FACE
President
AACE



Claudia Graham, PhD, MPH
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Medtronic Diabetes

¹ Diabetes In America 2nd Edition, National Diabetes Data Group, National Institutes of Health (NIH), National Institute of Diabetes and Digestive and Kidney Diseases, NIH Publication No. 95-1468. 1995.

² American Diabetes Association. (2003) Economic Costs of Diabetes in the U.S. in 2002. *Diabetes Care*. 26:917-932.

³ Diabetes Control and Complications Trial Research Group. (1993) The effect of intensive treatment of diabetes on the development and progression of long-term complications in insulin-dependent diabetes mellitus. *N Engl J Med*. 329:977-86.

⁴ UK Prospective Diabetes Study (UKPDS) Group. (1998) Intensive blood-glucose control with sulphonylureas or insulin compared with conventional treatment and risk of complications in patients with type 2 diabetes (UKPDS 33). *Lancet*. 352:837-853.

⁵ Roper Starch Diabetes Global Program, 2003 Diabetes Study, NOP World Health.

⁶ Bertoni, A. et al. (2004) Excess mortality related to diabetes mellitus in elderly Medicare beneficiaries. *Annals of Epid*. 14:362-367.

⁷ JDRF General Diabetes Fact Sheet. www.jdrf.org. Accessed June 23, 2004.

⁸ McCall, D. (2004) Are low-income elderly patients at risk for poor diabetes care? *Diabetes Care*. 27(5):1060-1065.

⁹ American Diabetes Association, www.diabetes.org. Accessed June 14, 2004.

¹⁰ United States Renal Data System, 2003 Annual Data Report, Reference Table K.1.

¹¹ Industry estimates based on insulin pump sales data. 2004.

¹² National Conference of State Legislatures, <http://www.ncsl.org/programs/health/diabetes.htm>. Accessed June 21, 2004.

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- ¹³ Mculloch D. (2000) Managing diabetes for improved health and economic outcomes. *American Journal of Managed Care*. 6(Supplement 21):S1089-95.
- ¹⁴ Herman, WH and Eastman, RC. (1998) The effects of treatment on the direct costs of diabetes. *Diabetes Care*. 21(Supplement 3):C19-24.
- ¹⁵ Campbell, LK and Campbell, RK. (1997) Cost drivers in diabetes care: the problems they present and potential solutions. *Clinical Therapeutics*. 19(3):540-58.
- ¹⁶ Herman WH, Eastman RC. (1998) The effects of treatment on the direct costs of diabetes. *Diabetes Care*. 21(Supplement 3):C19-C24.
- ¹⁷ Eastman RC, Javitt JC, Herman WH, et al. (1997) Model of complications of NIDDM. II. Analysis of the health benefits and cost-effectiveness of treating NIDDM with the goal of normoglycemia. *Diabetes Care*. 20:735-744.
- ¹⁸ Diabetes Control and Complications Trial Research Group. The effect of intensive treatment of diabetes on the development and progression of long-term complications in insulin-dependent diabetes mellitus. *N Engl J Med*. 1993. 329:977-86.
- ¹⁹ UKPDS Group. (1998) Intensive blood-glucose control with sulphonylureas or insulin compared with conventional treatment and risk of complications in patients with type 2 diabetes (UKPDS 33). *Lancet*. 352:837-853.
- ²⁰ UKPDS Group. (1998) Effect of intensive blood-glucose control with metformin on complications in overweight patients with type 2 diabetes (UKPDS 34) [published erratum appears in *Lancet*. 1998. 352:1557]. *Lancet*. 352:854-865.
- ²¹ Ohkubo Y, Kishikawa H, Araki E, et al. (1995) Intensive insulin therapy prevents the progression of diabetic microvascular complications in Japanese patients with non-insulin-dependent diabetes mellitus: a randomized prospective 6-year study. *Diabetes Res Clin Pract*. 28:103-117.
- ²² Hellman R, Regan J, Rosen H. (1997) Effect of intensive treatment of diabetes on the risk of death or renal failure in NIDDM and IDDM. *Diabetes Care*. 20:258-264 and AACE Consensus Development Conference on Guidelines for Glycemic Control. *Endocr Pract. Suppl. Nov/Dec 2001*.
- ²³ Davidson M. (2003) The Case for “Outsourcing” Diabetes Care. *Diabetes Care*. 26:1608-1612.
- ²⁴ The American Association of Clinical Endocrinologists Medical Guidelines for the Management of Diabetes Mellitus: The AACE System of Intensive Diabetes Self-Management—2002 Update. *Endocr Pract*. Jan/Feb 2002. 8:(Supplement 1).
- ²⁵ The ADA currently is advocating for NCQA to adopt the less than 7% HbA1C measure in the HEDIS® program.