MEDCAC Meetings

11/17/2010 - On Label and Off-Label Use of Autologous Cellular Immunotherapy Treatment of Metastatic Prostate Cancer

Issue

The Centers for Medicare and Medicaid Services (CMS) has called this meeting to consider the currently available evidence regarding the impact of labeled and unlabeled use of autologous cellular immunotherapy treatment on health outcomes of patients with metastatic prostate cancer.

As described on the FDA website at http://www.fda.gov/BiologicsBloodVaccines/CellularGeneTherapyProducts/ApprovedProducts/ucm213559.htm, "PROVENGE® (Sipuleucel T, APC8015) is an autologous cellular immunotherapy product consisting of peripheral blood mononuclear cells (PBMCs) obtained from patients by leukapheresis and activated *in vitro* with a recombinant fusion protein (prostatic acid phosphatase fused with GM-CSF). FDA will require the sponsor to complete a post marketing study to evaluate the risk of stroke in patients who receive sipuleucel-T."

Provenge® has FDA approved labeling for the treatment of asymptomatic or minimally symptomatic metastatic castrate resistant (hormone refractory) prostate cancer.

Actions Taken

August 31, 2010	Announced meeting.
	Those interested can register at: http://www.cms.gov/apps/events/event.asp?id=610&Kw=&Mh=NoMonth&cboOrder=date&Yr=NoYear&type=2
September 24, 2010	Posted FR Notice and questions to panel.
October 26, 2010	We will be broadcasting the meeting via Webinar. You must register for the Webinar portion of the meeting at https://webinar.cms.hhs.gov/txmetaprostatemedcac1117/event/registration.html

November 10, 2010	Posted technology assessment.
November 15, 2010	Posted agenda, roster and speaker list.
November 18, 2010	Posted scoresheet [PDF, 35KB] from meeting.

Federal Register Notice

Agenda

Agenda
Medicare Evidence Development & Coverage Advisory Committee
November 17, 2010
7:30 AM – 4:30 PM
CMS Auditorium

Clifford Goodman, PhD, Chair
Saty Satya-Murti, MD, Vice Chair
James Rollins, MD, Division Director, Division of Items and Devices, Coverage and Analysis Group
Maria Ellis, Executive Secretary

7:30 – 8:00 AM	Registration
8:00 – 8:15 AM	Opening Remarks—**Maria Ellis / James Rollins, MD / Clifford Goodman, PhD**
8:15 - 8:25 AM	CMS Presentation of Voting Questions – **Lori Paserchia, MD**
8:25 – 8:50 AM	**James L. Gulley, MD, PhD, FACP,** Director, Clinical Trials Group, Laboratory of Tumor, Immunology and Biology, Principal Investigator, Medical Oncology Branch, Center for Cancer Research, National Cancer Institute, National Institutes of Health
8:50 – 9:50 AM	TA Presentation: **David Mark, MD, MPH,** Senior Scientist, Blue Cross Blue Shield Association, Technology Evaluation Center
9:50 – 10:05 AM	BREAK
10:05 – 10:50 AM	Scheduled Public Comments (Refer to Speaker List)

Public attendees, who have contacted the executive secretary prior to the meeting, will address the panel and present information relevant to the agenda. Speakers are asked to state whether or not they have any financial involvement with manufacturers of any products being discussed or with their competitors and who funded their travel to this meeting.

10:50 – 11:05 AM	Open Public Comments

Public Attendees who wish to address the panel will be given that opportunity

11:05 – 12:00 PM	Questions to Presenters
12:00 – 1:00 PM	LUNCH (on your own)
1:00 – 2:00 PM	Initial Open Panel Discussion: Dr. Goodman
2:00 – 3:00 PM	Formal Remarks and Voting Questions

The Chairperson will ask each panel member to state his or her position on the voting questions

3:00 – 4:00 PM	Final Open Panel Discussion: Dr. Goodman
4:00 – 4:30 PM	Closing Remarks/Adjournment: Dr. Rollins & Dr. Goodman

Panel Voting Questions

MEDCAC –November 17, 2010
DRAFT QUESTIONS
Autologous Cellular Immunotherapy Treatment of Metastatic Prostate Cancer

FDA Label -
PROVENGE is an autologous cellular immunotherapy indicated for the treatment of asymptomatic or minimally symptomatic metastatic castrate resistant (hormone refractory) prostate cancer.

Voting Questions

For all voting questions, the health outcomes of interest are: overall survival, control of disease-related symptoms, and the avoidance or minimization of the burdens to patients associated with anticancer therapy. The comparator is the management that the patient would otherwise have received.

For the voting questions, use the following scale identifying level of confidence - with 1 being the lowest or no confidence and 5 representing a high level of confidence.

1 Low confidence	*2*	*3 Intermediate confidence*	*4*	*5 High confidence*

1. **How confident are you that there is adequate evidence to determine whether or not the use of autologous cellular immunotherapy treatment of asymptomatic or minimally**

symptomatic metastatic castrate resistant prostate cancer significantly improves:

a. **Overall survival?**

1	2	3	4	5

b. **Control of disease-related symptoms?**

1	2	3	4	5

c. **Avoidance or minimization of the burdens associated with anticancer therapy while maintaining overall survival and control of disease-related symptoms?**

1	2	3	4	5

Note: Questions 2 -6 should be addressed only for those outcomes under question 1 where the panel is confident that there is at least intermediate confidence (mean vote of 2.5) that there is adequate evidence to make the determination of improvement.

2. **How confident are you that there is adequate evidence to conclude that autologous cellular immunotherapy treatment significantly improves overall survival in patients with asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer?**

1	2	3	4	5

3. **How confident are you that there is adequate evidence to conclude that autologous cellular immunotherapy treatment significantly improves control of disease-related symptoms in patients with asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer?**

1	2	3	4	5

4. **How confident are you that there is adequate evidence to conclude that autologous cellular immunotherapy treatment significantly improves the avoidance of the treatment burdens (e.g., access, delivery, or side-effects) associated with anticancer therapy in patients with asymptomatic or minimally symptomatic metastatic castrate resistant prostate cancer?**

1	2	3	4	5

5. **How confident are you that these conclusions are generalizable to unlabeled use in:**

   a. **Patients whose prostate cancer has not metastasized?**

1	2	3	4	5

   b. **Patients who have metastatic, castrate resistant disease and symptoms more severe than minimally symptomatic?**

1	2	3	4	5

   c. **Patients who have metastatic prostate cancer but who have not failed hormonal therapy?**

1	2	3	4	5

6. **How confident are you that these conclusions are generalizable to:**

   a. **Community based settings?**

1	2	3	4	5

   b. **Patients belonging to demographic groups that may have been under-represented in the enrolled clinical trial populations?**

1	2	3	4	5

Discussion Questions

7. **Do you believe that there is adequate evidence to identify patients who are more likely or less likely to respond favorably to autologous cellular immunotherapy treatment based on pretreatment evaluation of any of the following factors?**

   a. **Site(s) or number of metastasis(es) as detected by imaging studies**
   b. **Gleason score**
   c. **Alkaline phosphatase (U/L)**
   d. **Hemoglobin (g/dL)**

e. **Serum LDH (U/L)**
f. **Serum PSA (ng/mL)**
g. **Pain associated with metastatic, castrate-resistant prostate cancer**
h. **Other**

8. **What significant evidence gaps exist regarding the health outcomes attributable to autologous cellular immunotherapy treatment?**
   a. **For the FDA-labeled indication?**
   b. **For off-label uses?**

9. **What clinical study designs would adequately address any evidence gaps?**

Download scoreshet [PDF, 35KB]

Directions to CMS

Recommended Hotels

Roster

Clifford Goodman, PhD **CHAIR**
Senior Vice President
The Lewin Group

Saty Satya-Murti, MD, FAAN VICE CHAIR
Health Policy Consultant

Helen Darling, MA
National Business Group on Health

Roger Dmochowski, MD
Department of Urology
Vanderbilt University

Dale Fuller, MD
Radiation Oncologist
Texas Oncology, PA

Karl Matuszewski, MS, PharmD
Vice President, Editor-in-Chief
Elsevier/Gold Standard

David M. Mintzer, MD
Chief
Section of Hematology and Medical Oncology Medical Director
Pain and Supportive Care Services
Pennsylvania Hospital
Clinical Associate
Professor of Medicine
University of Pennsylvania

Pearl Moore, RN, MN, FAAN
Adjunct Assistant Professor

Robert L. Steinbrook, MD
Adjunct Associate Professor of Medicine
Community and Family Medicine
Dartmouth Medical School

Industry Representative
G. Gregory Raab, PhD
Health Policy Consultant
Raab Associates

Guest Panel Members
Ravi A. Madan, MD
Assistant Clinical Investigator
Laboratory of Tumor Immunology and Biology and Medical Oncology Branch
National Cancer Institute
Nation Institute of Health

Mitchell Howard Sokoloff, MD, FACS
Professor of Surgery and Chief of Urology
Division of Urology
Department of Surgery
University of Arizona College of Medicine

Invited Guest Speaker
James L. Gulley, MD, PhD, FACP
Director
Clinical Trials Group, Laboratory of Tumor Immunology and Biology
Principal Investigator
Medical Oncology Branch

University of Pittsburgh School of Nursing

Louis Potters, MD, FACR
Chairman
Department of Radiation Medicine
North Shore – Long Island Jewish Health

Kevin Schulman, MD, MBA
Professor of Medicine
Duke University School of Medicine

Center for Cancer Research
National Cancer Institute
National Institutes of Health

CMS Liaison
James Rollins, MD
Division Director
Division of Items and Devices
Coverage and Analysis Group

Executive Secretary
Maria Ellis
Coverage and Analysis Group

Speaker List

Medicare Evidence Development & Coverage Advisory Committee
November 17, 2010

SPEAKER LIST

5 MINUTES PER SPEAKER

- **Paul F. Schellhammer, MD,** Professor of Urology, Eastern Virginia Medical School -
  Representing: American Urological Association
- **Brad Loncar,** Lenexa, Kansas
- **James J. Kiefert, MD,** Board Chairman Emeritus, Us TOO, International
- **Daniel P. Petrylak, MD,** Professor of Medicine, Columbia University Medical Center
- **Saurabh Aggarwal, PhD,** Healthcare Consultant, Bethesda, Maryland
- **Mark Scholz, MD,** Medical Director, Prostate Oncology Specialists, Inc., Marina Del Rey, California
- **Thair Phillips,** President, Retire Safe
- **Mark W. Frohlich, MD,** Chief Medical Officer, Dendreon Corporation
- **Philip Kantoff, MD**, Professor of Medicine, Harvard Medical School, Director, Genitourinary Oncology, Chief Clinical Research Officer and Chief Division of Solid Tumor Oncology, Dana-Farber Cancer Institute

Technology Assessment

Outcomes of Sipuleucel-T Therapy

Associated NCA

Autologous Cellular Immunotherapy Treatment of Metastatic Prostate Cancer (CAG-00422N)

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