Webinar: CAR-T Approval: What’s Next

What these historic approvals mean for the patient community, as well as how this sector will approach the related scientific, clinical, policy and business issues.
Today’s Commentators

Panelists:
- Heidi Hagen, Chief Strategy Officer & Co-Founder, Vineti (moderator)
- Gwen Nichols, M.D., Chief Medical Officer, Leukemia & Lymphoma Society
- Bruce Thompson, Ph.D., Senior Scientific Director, Therapeutic Products Program, Fred Hutchinson Cancer Center
- Michael Werner, Executive Director, Alliance for Regenerative Medicine; Partner, Holland & Knight

CAR-T Primer:
- Patricia Reilly, Vice President Intelligence Alliances & Unification, Informa
Primer: What is CAR-T?

How CAR-T Works:

- Uses the body’s own defense mechanism, the immune system, to fight cancer.
  - Immunotherapy improves the body’s ability to detect and kill cancer cells
  - Engineering patients’ own T cells to recognize and attack cancer cells:
    - Chimeric antigen receptor: CAR T-cell therapy
- FDA approved Novartis’s CAR T-cell-based gene therapy Kymriah on August 30, 2017.
  - Pediatric/young adult B-cell relapsed/refractory acute lymphoblastic leukemia patients
- FDA approved Gilead’s CAR T-cell-based therapy Yescarta on October 18, 2017.
  - Adults relapsed/refractory large B-cell lymphoma after two or more lines of systemic therapy
- CAR T-cell therapies and other cell-based immunoncology technologies will transform the standard of care for many oncological conditions.

Information & graphic provided by the Leukemia & Lymphoma Society, www.lls.org
Heidi Hagen, Chief Strategy Officer & Co-Founder, Vineti
• Proof of concept for genetic engineering tools used in cell products, creates a platform for other engineering tools such as CRISPR, TALENs, or ZFNs in cell therapies
• Opens the door for more investment funding in next-generation personalized medicine
• Enables patients to be engaged personally in fighting their disease; a sense of control

Gwen Nichols, M.D., Chief Medical Officer, Leukemia & Lymphoma Society
• New live-saving option for blood cancer patients
• LLS has long recognized the promise and potential of this technology; early funder of CAR T-cell immunotherapy, has invested $40+ million over the past 20 years towards this research

Bruce Thompson, Ph.D., Senior Scientific Director, Therapeutic Products Program, Fred Hutchinson Cancer Center
• Tremendous impact on patients, on R&D community
• Highlights importance of logistics, manufacturing in broadening patient access

Michael Werner, Executive Director, Alliance for Regenerative Medicine; Partner, Holland & Knight
• Game-changing therapeutic option, many lives will be saved as a result
• Opens the door for other cell- and gene-based, durable / potentially curative approaches to come to market
• Highlights importance of supportive, innovative reimbursement models
Opportunities & Challenges

**Heidi Hagen, Chief Strategy Officer & Co-Founder, Vineti**
- **Opportunity**: Commercial success in these cell therapies will pave the way to more efficient and cost-effective means to order, manufacture and deliver personalized medicines of all kinds
- **Challenge**: Creating a value based approach to pricing and reimbursement systems

**Gwen Nichols, M.D., Chief Medical Officer, Leukemia & Lymphoma Society**
- **Opportunity**: Long-awaited medical advance now available to more patients
- **Challenge**: Bringing this (and other new) technology to additional patient populations & indications

**Bruce Thompson, Ph.D., Senior Scientific Director, Therapeutic Products Program, Fred Hutchinson Cancer Center**
- **Opportunity**: Bridging R&D and patient communities
- **Challenge**: How to improve manufacturing processes, create up/down scalable platforms, logistics

**Michael Werner, Executive Director, Alliance for Regenerative Medicine; Partner, Holland & Knight**
- **Opportunity**: More patients now have a powerful new tool to fight cancer
- **Challenge**: Ensuring a supportive regulatory and reimbursement environment to facilitate development and broaden patient access
Creating a Supportive Environment

Heidi Hagen, Chief Strategy Officer & Co-Founder, Vineti
• Providing patient engagement tools that create greater access to new classes of therapies for deadly diseases
• Means to allow their caregivers more information and control over their care
• Find ways to expand the care provider network capable of prescribing these new products

Gwen Nichols, Chief Medical Officer, M.D., Leukemia & Lymphoma Society
• Stakeholder education outreach to drive awareness, acceptance, action
• R&D funding and legislative support

Bruce Thompson, Ph.D., Senior Scientific Director, Therapeutic Products Program, Fred Hutchinson Cancer Center
• Building the infrastructure needed to support development & implementation of industry-wide standards, manufacturing and logistics practices

Michael Werner, Executive Director, Alliance for Regenerative Medicine; Partner, Holland & Knight
• Given transformative nature and immense value of these products, new reimbursement / pricing models are needed
• Ensure providers & institutions are not disincentivized due to upfront costs
• Streamline and converge regulatory pathways to speed safe and effective products to patients in need
Audience Q&A