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.

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Today's Commentators

CAR-T Primer:

- ***Patricia Reilly**, Vice President Intelligence Alliances & Unification, Informa*

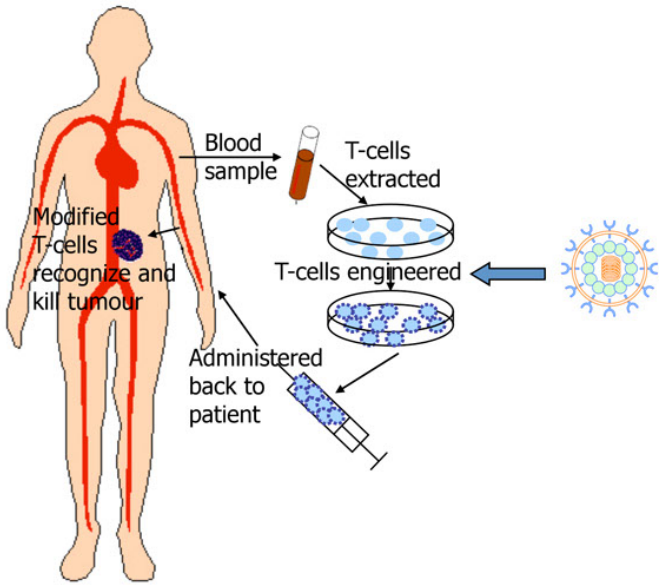
Panelists:

- ***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*

Primer: What is CAR-T?

- ***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 immuno-oncology technologies will transform the standard of care for many oncological conditions.***

How CAR-T Works:



Information & graphic provided by the Leukemia & Lymphoma Society, www.lls.org

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Impact of CAR-T Approval

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 theses 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

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