[CAR T-Cell Therapy: High Risk, High Reward Part 1](http://blog.fisherbioservices.com/car-t-cell-therapy-high-risk-high-reward-part-1)

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Of all the advances in medicine recently, [cellular therapy](http://www.fisherbioservices.com/market-solutions/cell-therapy) is perhaps the most intriguing. It is a fast-paced, ever changing market that tests the boundaries of innovation and has forever changed the way we approach certain diseases. However, this type of medical discovery comes with its fair share of risk, but when all goes according to plan, the rewards can be great.

As the Director of the Advanced Cell Therapy Lab at Yale University and Scientific Adviser to Thermo Fisher Scientific, Dr. Alexey Bersenev knows this very well. In this two-part guest blog series, Dr. Bersenev will discuss the promise of CAR T-cell therapy as well as some of the unresolved questions.



**CAR T-Cell Therapy: High Risk, High Reward Part 1**

For those of us working in the cell therapy industry, we are all very impressed by the latest “hot thing” - Chimeric Antigen Receptor (CAR) T-cells. Why is it hot right now? Below are my top five reasons why CAR T-cell therapies are taking the industry by storm:

1. CAR T-cell therapy has demonstrated the highest efficacy rates the cell therapy field has ever seen. This includes up to [90%](http://www.nejm.org/doi/full/10.1056/NEJMoa1407222#t=article) in pediatric and[93%](https://www.jci.org/articles/view/85309) complete remissions in adult B-ALL, as well as up to [53%](http://jco.ascopubs.org/content/early/2014/08/25/JCO.2014.56.2025.short) of complete remissions in lymphomas and CLL.
2. Unlike other experimental or standard therapies, CAR T-cells can potentially (meaning not in all patients and not in all conditions) be curative. Additionally, as of today, all highly efficacious results were reported in such “no options” categories of patients as “relapsed/ refractory” (r/r).
3. Despite many differences in gene construct design, manufacturing and clinical protocols, basic principles of CAR T-cell therapies are remarkably reproducible across clinical centers. So, it is spreading like a fire all over the world!
4. Biotech and Pharma are buying it! Every major biopharmaceutical company now has CAR T-cell therapy assets (Amgen, Shire, Pfizer, Regeneron, Johnson & Johnson, Celgene – to name a few). Several dozen startups with up to $4 billion of market capitalization (Juno Therapeutics capitalization on a peak exceeded $4B), 1 billion-sized deals (Juno Therapeutics and Celgene in 2015) and numerous strategic acquisitions have been seen in the last 3 years.
5. There is great regulatory support for developers. “Breakthrough Therapy” designations, granted by US FDA to such CAR T-cell therapy developers as Novartis, Juno Therapeutics and Kite Pharma will allow faster review of all their regulatory submissions by the agency. Another sign of great regulatory support in the US is the FDA’s proposal for a [CAR T-cell therapy database](http://celltrials.info/2016/03/16/fda-cart-database/). Expedited reviews by FDA and regulatory approval of pivotal trials with number of patients below 100-120 (due to high efficacy) will allow companies to get to market as soon as 5-6 years from the beginning of commercial development. Thus, Novartis and Kite Pharma are expecting to get the first CAR T-cell therapy products on the market as early as next year.

As you can see from the references above, there are compelling reasons regarding the buzz that surrounds CAR T-cell therapy. However, despite all this success, there are still many unresolved questions and issues. Stay tuned for Pt. 2 of my blog series where I’ll explore some of these challenges.