



## Positive Pivotal Data for Libtayo® (cemiplimab) Monotherapy in Locally Advanced Basal Cell Carcinoma Featured as a Late-breaking Presentation at ESMO

September 18, 2020

TARRYTOWN, N.Y. and PARIS, Sept. 18, 2020 /PRNewswire/ --

**Libtayo is the first investigational medicine to show a clinical benefit in advanced basal cell carcinoma following treatment with a hedgehog inhibitor in a prospective trial**

**31% objective response rate seen in trial patients, and an estimated 85% of responses were ongoing at one year**

Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) and Sanofi today announced the presentation of positive results from the pivotal Phase 2 trial for the PD-1 inhibitor Libtayo® (cemiplimab) in patients with locally advanced basal cell carcinoma (BCC) who had progressed on or were intolerant to hedgehog inhibitor (HHI) therapy. The data were shared in a late-breaking presentation at the European Society for Medical Oncology (ESMO) Virtual Congress 2020, and form the basis of regulatory submissions, including in the U.S. and European Union.

"Advanced basal cell carcinoma can be an unrelenting, highly disfiguring disease, and there are no approved treatment options once a patient progresses on or becomes intolerant to hedgehog inhibitors," said Alexander Stratigos, M.D., Professor of Dermatology at the University of Athens Medical School at Andreas Sygros Hospital and a trial investigator. "This is the first time a prospective trial of an investigational medicine has shown a clinical benefit in this patient population, and the Libtayo data provide hope for this difficult-to-treat cancer."

Per independent central review, the objective response rate (ORR) was 31% among Libtayo-treated patients (n=84; 95% confidence interval [CI]: 21-42%), with a median follow-up of 15 months (range: 1-25 months). This included a 6% (n=5) complete and 25% (n=21) partial response rate. This is an increase from the ORR [shared](#) in May and includes two responses that were confirmed after the initial data analysis. Responses were seen regardless of baseline PD-L1 expression in tumor cells.

As of data cut-off, the median duration of response and median overall survival had not yet been reached. At one-year, 85% of responses were ongoing (95% CI: 61-95%), the probability of progression-free survival was 57% (95% CI: 44-67%), and the probability of overall survival was 92% (95% CI: 84-97%), according to Kaplan-Meier estimates.

No new Libtayo safety signals were observed. The most common treatment-related adverse events (AEs) were fatigue (25%, n=21), pruritus (14%, n=12) and asthenia (14%, n=12). Grade 3 or higher treatment-related AEs that occurred in at least 2 patients were colitis (5%, n=4), fatigue and adrenal insufficiency (2%, n=2 each). Fourteen patients (17%) discontinued treatment due to treatment-emergent AEs.

Libtayo was invented using Regeneron's *VelocImmune*® technology that utilizes a proprietary genetically-engineered mouse platform endowed with a genetically-humanized immune system to produce optimized fully-human antibodies. *VelocImmune* technology has been used to create multiple antibodies including Dupixent® (dupilumab), Praluent® (alirocumab) and Kevzara® (sarilumab), which are approved in multiple countries around the world. Regeneron previously used these technologies to rapidly develop a treatment for Ebola virus infection, which is currently under review by the FDA, and to create REGN-COV2, a potentially preventative and therapeutic medicine for COVID-19.

Libtayo is being jointly developed by Regeneron and Sanofi under a global collaboration agreement. The use of Libtayo to treat advanced BCC is investigational and has not been fully evaluated by any regulatory authority.

### About the Pivotal BCC Trial

In this ongoing, global Phase 2 trial, two cohorts of patients were studied: locally advanced BCC and metastatic BCC. All patients received Libtayo 350 mg intravenously every three weeks for up to 93 weeks or until disease progression. The primary endpoint is ORR, and key secondary endpoints include overall survival, progression free survival, duration of response, safety and tolerability. The median duration of response and median overall survival were estimated using Kaplan-Meier analyses. Detailed interim metastatic BCC data are planned for presentation at an upcoming medical meeting.

### About BCC

BCC is a common type of non-melanoma skin cancer. While the vast majority of BCCs are caught early and easily cured with surgery and radiation, a small proportion of tumors can become advanced and penetrate deeper into surrounding tissues (locally advanced) or spread to other parts of the body (metastatic), which is more difficult to treat. In the U.S. alone, approximately 2 million new cases of BCC will be diagnosed every year, 20,000 U.S. patients will have advanced BCC and 3,000 patients will die of this disease.

### About Libtayo

Libtayo is a fully-human monoclonal antibody targeting the immune checkpoint receptor PD-1 on T-cells. By binding to PD-1, Libtayo has been shown to block cancer cells from using the PD-1 pathway to suppress T-cell activation.

Libtayo is the first immunotherapy approved in the U.S., EU and other countries for adults with metastatic cutaneous squamous cell carcinoma (CSCC) or locally advanced CSCC who are not candidates for curative surgery or curative radiation. In the U.S., the generic name for Libtayo in its approved indication is cemiplimab-rwlc, with rwlc as the suffix designated in accordance with Nonproprietary Naming of Biological Products Guidance for Industry issued by the U.S. Food and Drug Administration. Outside of the U.S., the generic name for Libtayo in its approved indication is cemiplimab.

The extensive clinical program for Libtayo is focused on difficult-to-treat cancers. In skin cancer, this includes trials in adjuvant and neoadjuvant CSCC in addition to the pivotal trial in advanced BCC. Libtayo is also being investigated in pivotal trials in NSCLC and cervical cancer, as well as in trials combining Libtayo with either conventional or novel therapeutic approaches for both solid tumors and blood cancers. These potential uses are investigational, and their safety and efficacy have not been evaluated by any regulatory authority.

## **IMPORTANT SAFETY INFORMATION AND INDICATION FOR U.S. PATIENTS**

### **What is Libtayo?**

Libtayo is a prescription medicine used to treat people with a type of skin cancer called cutaneous squamous cell carcinoma (CSCC) that has spread or cannot be cured by surgery or radiation.

It is not known if Libtayo is safe and effective in children.

### **What is the most important information I should know about Libtayo?**

Libtayo is a medicine that may treat a type of skin cancer by working with your immune system. Libtayo can cause your immune system to attack normal organs and tissues in any area of your body and can affect the way they work. These problems can sometimes become severe or life-threatening and can lead to death. You can have more than one problem at the same time. These problems may happen anytime during treatment or even after your treatment has ended.

**Call or see your healthcare provider right away if you develop any symptoms of the following problems or these symptoms get worse:**

- **Lung problems (pneumonitis).** Signs and symptoms of pneumonitis may include new or worsening cough, shortness of breath, and chest pain.
- **Intestinal problems (colitis)** that can lead to tears or holes in your intestine. Signs and symptoms of colitis may include diarrhea (loose stools) or more frequent bowel movements than usual; stools that are black, tarry, sticky or that have blood or mucus; and severe stomach-area (abdomen) pain or tenderness.
- **Liver problems (hepatitis).** Signs and symptoms of hepatitis may include yellowing of your skin or the whites of your eyes, severe nausea or vomiting, pain on the right side of your stomach area (abdomen), drowsiness, dark urine (tea colored), bleeding or bruising more easily than normal, and feeling less hungry than usual.
- **Hormone gland problems** (especially the adrenal glands, pituitary, thyroid and pancreas). Signs and symptoms that your hormone glands are not working properly may include headaches that will not go away or unusual headaches, rapid heartbeat, increased sweating, extreme tiredness, weight gain or weight loss, dizziness or fainting, feeling more hungry or thirsty than usual, hair loss, feeling cold, constipation, deeper voice, very low blood pressure, urinating more often than usual, nausea or vomiting, stomach-area (abdomen) pain, and changes in mood or behavior, such as decreased sex drive, irritability, or forgetfulness.
- **Kidney problems**, including nephritis and kidney failure. Signs of these problems may include decrease in your amount of urine, blood in your urine, swelling in your ankles, and loss of appetite.
- **Skin problems.** Signs of these problems may include rash, itching, skin blistering, and painful sores or ulcers in the mouth, nose, throat, or genital area.
- **Problems in other organs.** Signs of these problems may include headache, tiredness or weakness, sleepiness, changes in heartbeat (such as beating fast, seeming to skip a beat, or a pounding sensation), confusion, fever, muscle weakness, balance problems, nausea, vomiting, stiff neck, memory problems, seizures (encephalitis), swollen lymph nodes, rash or tender lumps on skin, cough, shortness of breath, vision changes, or eye pain (sarcoidosis), seeing or hearing things that are not there (hallucinations), severe or persistent muscle pain, severe muscle weakness, low red blood cells (anemia), bruises on the skin or bleeding, and changes in eyesight.
- **Rejection of a transplanted organ.** Your doctor should tell you what signs and symptoms you should report and monitor you, depending on the type of organ transplant that you have had.
- **Infusion (IV) reactions that can sometimes be severe and life-threatening.** Signs of these problems may include chills or shaking, itching or rash, flushing, shortness of breath or wheezing, dizziness, fever, feeling of passing out, back or neck pain, and facial swelling.

**Getting medical treatment right away may help keep these problems from becoming more serious.**

Your healthcare provider will check you for these problems during your treatment with Libtayo. Your healthcare provider may treat you with corticosteroid or hormone replacement medicines. Your healthcare provider may delay or completely stop treatment if you have severe side effects.

**Before you receive Libtayo, tell your healthcare provider about all your medical conditions, including if you:**

- have immune system problems such as Crohn's disease, ulcerative colitis, or lupus;
- have had an organ transplant;
- have lung or breathing problems;
- have liver or kidney problems;
- have diabetes;
- are pregnant or plan to become pregnant; Libtayo can harm your unborn baby

#### **Females who are able to become pregnant:**

- Your healthcare provider will give you a pregnancy test before you start treatment.

- You should use an effective method of birth control during your treatment and for at least 4 months after your last dose of Libtayo. Talk with your healthcare provider about birth control methods that you can use during this time.
- Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with Libtayo.
- are breastfeeding or plan to breastfeed. It is not known if Libtayo passes into your breast milk. Do not breastfeed during treatment and for at least 4 months after the last dose of Libtayo.

**Tell your healthcare provider about all the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

The most common side effects of Libtayo include tiredness, rash, diarrhea, muscle or bone pain, and nausea. These are not all the possible side effects of Libtayo. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088. You may also report side effects to Regeneron Pharmaceuticals and Sanofi at 1-877-542-8296.

**Please see accompanying [full Prescribing Information, including Medication Guide](#).**

#### **About Regeneron Pharmaceuticals, Inc.**

Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for over 30 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to seven FDA-approved treatments and numerous product candidates in development, all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, pain, infectious diseases and rare diseases.

Regeneron is accelerating and improving the traditional drug development process through our proprietary *VelociSuite*<sup>®</sup> technologies, such as *VelocImmune* which uses unique genetically-humanized mice to produce optimized fully-human antibodies and bispecific antibodies, and through ambitious research initiatives such as the Regeneron Genetics Center, which is conducting one of the largest genetics sequencing efforts in the world.

For additional information about the company, please visit [www.regeneron.com](http://www.regeneron.com) or follow @Regeneron on Twitter.

#### **About Sanofi**

Sanofi is dedicated to supporting people through their health challenges. We are a global biopharmaceutical company focused on human health. We prevent illness with vaccines, provide innovative treatments to fight pain and ease suffering. We stand by the few who suffer from rare diseases and the millions with long-term chronic conditions.

With more than 100,000 people in 100 countries, Sanofi is transforming scientific innovation into healthcare solutions around the globe.

Sanofi, Empowering Life

#### **Regeneron Forward-Looking Statements and Use of Digital Media**

*This press release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the impact of SARS-CoV-2 (the virus that has caused the COVID-19 pandemic) on Regeneron's business and its employees, collaborators, and suppliers and other third parties on which Regeneron relies, Regeneron's and its collaborators' ability to continue to conduct research and clinical programs, Regeneron's ability to manage its supply chain, net product sales of products marketed by Regeneron and/or its collaborators (collectively, "Regeneron's Products"), and the global economy; the nature, timing, and possible success and therapeutic applications of Regeneron's Products and Regeneron's product candidates and research and clinical programs now underway or planned, including without limitation Libtayo<sup>®</sup> (cemiplimab); the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's product candidates and new indications for Regeneron's Products, such as Libtayo for the treatment of basal cell carcinoma, non-small cell lung cancer, adjuvant and neoadjuvant cutaneous squamous cell carcinoma, and cervical cancer (as well as in trials combining Libtayo with either conventional or novel therapeutic approaches for both solid tumors and blood cancers, as applicable); uncertainty of market acceptance and commercial success of Regeneron's Products and product candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary) on the commercial success of Regeneron's Products and product candidates; safety issues resulting from the administration of Regeneron's Products (such as Libtayo) and product candidates in patients, including serious complications or side effects in connection with the use of Regeneron's Products and product candidates in clinical trials; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's Products and product candidates; ongoing regulatory obligations and oversight impacting Regeneron's Products, research and clinical programs, and business, including those relating to patient privacy; the availability and extent of reimbursement of Regeneron's Products from third-party payers, including private payer healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payers and new policies and procedures adopted by such payers; competing drugs and product candidates that may be superior to, or more cost effective than, Regeneron's Products and product candidates; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; the ability of Regeneron to manufacture and manage supply chains for multiple products and product candidates; the ability of Regeneron's collaborators, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and product candidates; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its financial projections or guidance and changes to the assumptions underlying those projections or guidance; the potential for any license or collaboration agreement, including Regeneron's agreements with Sanofi, Bayer, and Teva Pharmaceutical Industries Ltd. (or their respective affiliated companies, as applicable), to be cancelled or terminated without any further product success; and risks associated with intellectual property of other parties and pending or future litigation relating thereto (including without limitation the patent litigation and other related proceedings relating to EYLEA<sup>®</sup> (afibercept) Injection, Dupixent<sup>®</sup> (dupilumab), and Praluent<sup>®</sup> (alirocumab)), other litigation and other proceedings and government*

investigations relating to the Company and/or its operations, the ultimate outcome of any such proceedings and investigations, and the impact any of the foregoing may have on Regeneron's business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2019 and its Form 10-Q for the quarterly period ended June 30, 2020. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update publicly any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (<http://newsroom.regeneron.com>) and its Twitter feed (<http://twitter.com/regeneron>).

### Sanofi Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates regarding the marketing and other potential of the product, or regarding potential future revenues from the product. Forward-looking statements are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates", "plans" and similar expressions. Although Sanofi's management believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Sanofi, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, unexpected regulatory actions or delays, or government regulation generally, that could affect the availability or commercial potential of the product, the fact that product may not be commercially successful, the uncertainties inherent in research and development, including future clinical data and analysis of existing clinical data relating to the product, including post marketing, unexpected safety, quality or manufacturing issues, competition in general, risks associated with intellectual property and any related future litigation and the ultimate outcome of such litigation, and volatile economic and market conditions, and the impact that COVID-19 will have on us, our customers, suppliers, vendors, and other business partners, and the financial condition of any one of them, as well as on our employees and on the global economy as a whole. Any material effect of COVID-19 on any of the foregoing could also adversely impact us. This situation is changing rapidly and additional impacts may arise of which we are not currently aware and may exacerbate other previously identified risks. The risks and uncertainties also include the uncertainties discussed or identified in the public filings with the SEC and the AMF made by Sanofi, including those listed under "Risk Factors" and "Cautionary Statement Regarding Forward-Looking Statements" in Sanofi's annual report on Form 20-F for the year ended December 31, 2019. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

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