HEMISPHERX BIOPHARMA INC
Form 10-K
March 30, 2018

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

[X] ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the fiscal year ended December 31, 2017

OR

[ ] TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from _____ to _____

Commission File No. 000-27072

HEMISPHERX BIOPHARMA, INC.

(Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation or organization)	52-0845822 (I.R.S. Employer Identification Number)

860 N. Orange Avenue, Suite B, Orlando, Florida (Address of principal executive offices)	32801 (Zip Code)

Registrant's telephone number, including area code: (215) 988-0080

Securities registered pursuant to Section 12(b) of the Act:

Common Stock, $.001 par value

Securities registered pursuant to Section 12(g) of the Act:

(Title of Each Class)

NONE

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes [ ] No [X]

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes [ ] No [X]

Indicate by check mark whether the registrant (1) has filed all reports to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes [X] No [ ]

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes [X] No [ ]

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. [ ]

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer or a smaller reporting company. See definition of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

[ ]	Large accelerated filer	[ ]	Accelerated filer
[ ]	Non-accelerated filer	[X]	Smaller reporting company
		[ ]	Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards pursuant to Section 13(a) of the Exchange Act. [ ]

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes [ ] No [X]

The aggregate market value of Common Stock held by non-affiliates at June 30, 2017, the last business day of the registrant's most recently completed second fiscal quarter was $14,478,834.

The number of shares of the registrant's Common Stock outstanding as of March 26, 2018 was 37,715,230.

DOCUMENTS INCORPORATED BY REFERENCE: None.

TABLE OF CONTENTS

CAUTIONARY STATEMENT REGARDING FORWARD-LOOKING STATEMENTS

Certain statements in this Annual Report on Form 10-K (the "Form 10-K"), including statements under "Item 1-Business," "Item 1A-Risk Factors" and "Item 3-Legal Proceedings" in PART I and "Item 7-Management's Discussion and Analysis of Financial Condition and Result of Operations" in PART II, constitute "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and the Private Securities Litigation Reform Act of 1995. Certain, but not necessarily all, of such forward-looking statements can be identified by the use of forward-looking terminology such as "believes", "expects", "may", "will", "should", or "anticipates" or the negative thereof or other variations thereon or comparable terminology, or by discussions of strategy that involve risks and uncertainties. Forward-looking statements reflect our views as of the date that they are made with respect to future events and are based on assumptions. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. We discuss many of these risks, uncertainties and other important factors in greater detail under the "Risk Factor" sections in this Form 10-K. We can give no assurances that any of the events anticipated by the forward-looking statements will occur or, if any of them do, what impact they will have on our business, results of operations and financial condition. New factors emerge from time to time, and it is not possible for us to predict which will arise. We cannot assess the impact of each factor on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements. All statements other than statements of historical fact included in this Form 10-K regarding our financial position, business strategy and plans or objectives for future operations are forward-looking statements.

Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties inherent in our business including, without limitation: our ability to adequately fund our projects as we will need additional funding to proceed with our objectives, the potential therapeutic effect of our products, the possibility of obtaining regulatory approval, our ability to find senior co-development partners with the capital and expertise needed to commercialize our products and to enter into arrangements with them on commercially reasonable terms, our ability to manufacture and sell any products, our ability to enter into arrangements with third party vendors, market acceptance of our products, our ability to earn a profit from sales or licenses of any drugs, our ability to discover new drugs in the future, changing market conditions, changes in laws and regulations affecting our industry, and issues related to our New Brunswick, New Jersey facility. We have disclosed that in February 2013, we received a Complete Response from the U.S. Food and Drug Administration (the "FDA") for our Ampligen® New Drug Application ("NDA") for Chronic Fatigue Syndrome Treatment, sometimes referred to as myalgic encephalomyelitis/chronic fatigue syndrome ("ME/CFS"), stating that we should conduct at least one additional clinical trial, complete various nonclinical studies and perform a number of data analyses. Accordingly, the remaining steps to potentially gain FDA approval of the Ampligen® NDA, the final results of these and other ongoing activities could vary materially from our expectations and could adversely affect the chances for approval of the Ampligen® NDA. These activities and the ultimate outcomes are subject to a variety of risks and uncertainties, including but not limited to risks that (i) the FDA may ask for additional data, information or studies to be completed or provided; and (ii) the FDA may require additional work related to the commercial manufacturing process to be completed or may, in the course of the inspection of manufacturing facilities, identify issues to be resolved. With regard to our NDA for Ampligen® to treat ME/CFS, as noted above, there are additional steps which the FDA has advised Hemispherx to take in our seeking approval. The final results of these and other ongoing activities, and of the FDA review, could

vary materially from Hemispherx' expectations and could adversely affect the chances for approval of the Ampligen® NDA. Any failure to satisfy the FDA’s requirements could significantly delay, or preclude outright, approval of our drugs for commercial sale in the United States.

We also have disclosed that, in August 2016, we received approval of our NDA from AdministracionNacional de Medicamentos, Alimentos y TecnologiaMedica ("ANMAT") for commercial sale of rintatolimod (U.S. tradename: Ampligen®) in the Argentine Republic for the treatment of severe ME/CFS. The product will be marketed by GP Pharm, our commercial partner in Latin America. We believe, but cannot assure, that this approval provides a platform for potential sales in certain countries within the European Union under regulations that support cross-border pharmaceutical sales of licensed drugs. In Europe, approval in a country with a stringent regulatory process in place, such as Argentina, should add further validation for the product as the Early Access Program as discussed below and underway in Europe in pancreatic cancer. ANMAT approval is only an initial, but important, step in the overall successful commercialization of our product. There are a number of actions that must occur before we could be able to commence commercial sales in Argentina. Commercialization in Argentina will require, among other things, an appropriate reimbursement level, appropriate marketing strategies, completion of manufacturing preparations for launch (including possible requirements for approval of final manufacturing) and we most likely will need additional funds to manufacture product at a sufficient level for a commercial launch. There are no assurances as to whether or when such multiple subsequent steps will be successfully performed to result in an overall successful commercialization and product launch. Approval of rintatolimod for ME/CFS in the Argentine Republic does not in any way suggest that the Ampligen® NDA in the United States or any comparable application filed in the European Union or elsewhere will obtain commercial approval.

We also have disclosed that, in May 2016, we entered into a five year agreement with myTomorrows, a Netherlands based company, for the commencement and management of an Early Access Program ("EAP") in Europe and Turkey (the "Territory") related to CFS. Pursuant to the agreement, myTomorrows, as our exclusive service provider and distributor in the Territory, is performing EAP activities. In January 2017, we announced that the EAP has been extended to pancreatic cancer patients beginning in the Netherlands. In June 2017, we signed an amendment to provide support services to Hemispherx with respect to the execution of the 511-Program ("511-Services") and that the 511-Services shall be rendered free of charge. In February 2018, we signed an amendment to extend the territory to cover Canada to treat pancreatic cancer patients, pending government approval. In March 2018, we signed an amendment to which myTomorrows will be our exclusive service provider for special access activities in Canada for the supply of Ampligen® for the treatment of ME/CFS. No assurance can be given that we can sufficiently supply product should we experience an unexpected demand for Ampligen® in our clinical studies, the commercial launch in Argentina or pursuant to the EAP.

Our overall objectives include plans to continue seeking approval for commercialization of Ampligen® in the United States and abroad as well as seeking to broaden commercial therapeutic indications for Alferon N Injection® presently approved in the United States and Argentina. We continue to pursue senior co-development partners with the capital and expertise needed to commercialize our products and to enter into arrangements with them on commercially reasonable terms. Our ability to commercialize our products, widen commercial therapeutic indications of Alferon N Injection® and/or capitalize on our collaborations with research laboratories to examine our products are subject to a number of significant risks and uncertainties including, but not limited to our ability to enter into more definitive agreements with some of the research laboratories and others that we are collaborating with, to fund and conduct additional testing and studies, whether or not such testing is successful or requires additional testing and meets the requirements of the FDA and comparable foreign regulatory agencies. We do not know when, if ever, our products will be generally available for commercial sale for any indication.

4

We outsource certain components of our manufacturing, quality control, marketing and distribution while maintaining control over the entire process through our quality assurance and regulatory groups. We cannot provide any guarantee that the facility or our contract manufacturer will necessarily pass an FDA pre-approval inspection for Alferon® manufacture.

The production of new Alferon® API inventory will not commence until the validation phase is complete. While the facility is approved by FDA under the Biological License Application ("BLA") for Alferon®, this status will need to be reaffirmed by a successful Pre-Approval Inspection by the FDA prior to commercial sale of newly produced inventory product. If and when the Company obtains a reaffirmation of FDA BLA status and has begun production of new Alferon® API, it will need FDA approval as to the quality and stability of the final product to allow commercial sales to resume. We will need additional funds to finance the revalidation process in our facility to initiate commercial manufacturing, thereby readying ourselves for an FDA Pre-Approval Inspection. If we are unable to gain the necessary FDA approvals related to the manufacturing process and/or final product of new Alferon® inventory, our operations most likely will be materially and/or adversely affected. In light of these contingencies, there can be no assurances that the approved Alferon N Injection® product will be returned to production on a timely basis, if at all, or that if and when it is again made commercially available, it will return to prior sales levels.

We believe, and are investigating, Ampligen®'s potential role in enhancing the activity of influenza vaccines. While certain studies involving rodents, non-human primates (monkeys) and healthy human subjects indicate that Ampligen® may enhance the activity of influenza vaccines by conferring increased cross-reactivity or cross-protection, further studies will be required and no assurance can be given that Ampligen® will assist in the development of a universal vaccine for influenza or other viruses.

We do not undertake and specifically decline any obligation to publicly release the results of any revisions which may be made to any forward-looking statement to reflect events or circumstances after the date of such statements or to reflect the occurrence of anticipated or unanticipated events.

PART I

ITEM 1. Business

GENERAL

Hemispherx Biopharma, Inc. and its subsidiaries (collectively, “Hemispherx”, “Company”, “we” or “us”) are a specialty pharmaceutical company headquartered in Orlando, Florida and engaged in the development of new drug therapies based on natural immune system enhancing technologies for the treatment of viral and immune based disorders. We have established a strong foundation of laboratory, pre-clinical and clinical data with respect to the development of natural interferon and nucleic acids to enhance the natural antiviral defense system of the human body and to aid the development of therapeutic products for the treatment of certain chronic diseases.

Our flagship products include Alferon N Injection® and the experimental therapeutic Ampligen®. Alferon N Injection® is approved for a category of STD infection, and Ampligen® represents an experimental RNA being developed for globally important viral diseases and disorders of the immune system. Hemispherx’ platform technology includes components for potential treatment of various severely debilitating and life threatening diseases.

We operate a 30,000 sq. ft. facility in New Brunswick, NJ with the objective of producing Alferon® and Ampligen® upon FDA approval. As part of our objectives to achieve our commercial goals and increase stockholder value, we recently sold our main facility while obtaining a long term lease with a buy-back option on the facility. In addition, we sold an underutilized, unencumbered, and wholly owned building adjacent to our manufacturing facility site noted above. We do not believe that the sale of these buildings will have an impact on the production of our products. Please see “Part 2. Properties” below.

In February 2013, we received a Complete Response Letter (“CRL”) from the FDA for the NDA for Ampligen® for Chronic Fatigue Syndrome ("CFS") without further confirmatory clinical trials. Please see the discussion in "Our Products - Ampligen®" below for more detail.

We are committed to a focused business plan oriented toward finding senior co-development partners with the capital and expertise needed to commercialize the many potential therapeutic aspects of our experimental drugs and our FDA approved drug Alferon® N.

With keeping to our austerity plan to reserve capital we have relocated our principal executive office from a large expensive corporate space in center city Philadelphia to 860 N. Orange Avenue, Suite B, Orlando, FL 32801.

AVAILABLE INFORMATION

We file our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K pursuant to Section 13(a) or 15(d) of the Exchange Act electronically with the Securities and Exchange Commission, or SEC. The public may read or copy any materials we file with the SEC at the SEC's Public Reference Room at 100 F Street, NE, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. The SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding issuers that file electronically with the SEC. The address of that site is http://www.sec.gov.

You may obtain a free copy of our annual reports on Form 10-K, quarterly reports on Form 10-Q and current reports on Form 8-K and amendments to those reports on the day of filing with the SEC on our website on the World Wide Web at http://www.hemispherx.net under the Investor Relations tab for SEC Filings or by contacting the Investor Relations Department by calling 888-557-6480 or sending an e-mail message to ir@hemispherx.net.

OUR PRODUCTS

Our primary pharmaceutical product platform consists of our experimental compound, Ampligen®, and our FDA approved natural interferon product, Alferon N Injection®.

Ampligen®

Ampligen® is approved for sale in Argentina and is an experimental drug currently undergoing clinical development for the treatment of CFS in the United States of America. Over its developmental history, Ampligen® has received various designations, including Orphan Drug Product Designation (FDA), Treatment protocol (e.g., “Expanded Access” or “Compassionate” use authorization) with Cost Recovery Authorization (FDA) and “promising” clinical outcome recognition based on the evaluation of certain summary clinical reports (“AHRQ” or Agency for Healthcare Research and Quality). Ampligen® represents the first drug in the class of large (macromolecular) RNA (nucleic acid) molecules to apply for NDA review. Based on the results of published, peer reviewed pre-clinical studies and clinical trials, we believe that Ampligen® may have broad-spectrum anti-viral and anti-cancer properties.

We believe that nucleic acid compounds represent a potential new class of pharmaceutical products as they are designed to act at the molecular level for treatment of human diseases. There are two forms of nucleic acids, DNA and RNA. DNA is a group of naturally occurring molecules found in chromosomes, the cell’s genetic machinery. RNA is a group of naturally occurring informational molecules which orchestrate a cell’s behavior which, in turn, regulates the action of groups of cells, including the cells which compromise the body’s immune system. RNA directs the production of proteins and regulates certain cell activities including the activation of an otherwise dormant cellular defense against viruses and tumors. Our drug technology utilizes specifically-configured RNA. Our double-stranded RNA drug product, trademarked Ampligen®, is an experimental, unapproved drug in the United States, that is administered intravenously. Ampligen® has been assigned the generic name rintatolimod by the United States Adopted Names Council (USANC) and has the chemical designation poly(I):poly(C_{12}U).

Clinical trials of Ampligen® already conducted by us include studies of the potential treatment of CFS, Hepatitis B, HIV and cancer patients with renal cell carcinoma and malignant melanoma. All of these potential uses will require additional clinical trials to generate the safety and effectiveness data necessary to support regulatory approval.

In February 2013, we received a Complete Response Letter ("CRL") from the FDA for Ampligen® for CFS. In its CRL, the FDA communicated that Hemispherx should conduct at least one additional clinical trial, complete various nonclinical studies and perform a number of data analyses. We are actively engaged with the FDA, and have had several meetings in order to reach an agreement on the path forward. Until we reach an agreement with the FDA regarding the design of a study, we are unable to reasonably estimate the nature or costs necessary to obtain FDA clearance or anticipated completion dates of any additional clinical study or studies.

The FDA authorized an open-label treatment protocol, ("AMP-511"), allowing patient access to Ampligen® for treatment in an open-label safety study under which severely debilitated CFS patients have the opportunity to be on Ampligen® to treat this very serious and chronic condition. The data collected from the AMP-511 protocol through a consortium group of clinical sites provide safety information regarding the use of Ampligen® in patients with CFS. We are establishing an enlarged data base of clinical safety information which we believe will provide further documentation regarding the absence of autoimmune disease associated with Ampligen® treatment. We believe that continued efforts to understand existing data, and to advance the development of new data and information, will ultimately support our future filings for Ampligen® and/or the design of future clinical studies. In 2015, we engaged an independent certified public accountant to recalculate the cost per dose consistent with the current guidelines, utilizing the costs to produce a vial. In October 2016, the FDA granted our request to implement the new cost which was initiated during the quarter ended March 31, 2017.As of December 31, 2017, there are 17 patients participating in this open-label treatment protocol.

In August 2016, we received approval of our NDA from ANMAT for commercial sale of rintatolimod (U.S. tradename: Ampligen®) in the Argentine Republic for the treatment of ME/CFS. The product will be marketed by GP Pharm, our commercial partner in Latin America. There are a number of actions that must occur before we could be able to commence commercial sales in Argentina. Commercialization in Argentina will require, among other things, an appropriate reimbursement level, appropriate marketing strategies, completion of manufacturing preparations for launch (including possible requirements for approval of final manufacturing) and we most likely will need additional funds to manufacture product at a sufficient level for a commercial launch.

In May 2016, we entered into a five year agreement with myTomorrows, a Netherlands based company, for the commencement and management of an Early Access Program ("EAP") in Europe and Turkey (the "Territory") related to CFS. Subsequently we have made amendments to the original agreement in January 2017, June 2017, February 2018 and March 2018. Pursuant to the original agreement and the amendments myTomorrow's will manage all Early Access Programs and Special Access Programme's in Europe, Canada and Turkey to treat pancreatic cancer and ME/CFS patients. myTomorrows will also provide support services to Hemispherx with respect to the execution of the 511-cost recovery Program to treat ME/CFS patients in the USA.

In August, 2017 we announced that we have commenced full data analysis of an intranasal human safety study of Ampligen® plus FluMist® known as AMP-600. The study was previously closed, but the initiation of full data analysis awaited the FDA's evaluation of preliminary reports of blinded study findings. That evaluation was completed per formal notification from the FDA in August, 2017. Intranasal Ampligen was generally well-tolerated in the study.

Alferon N Injection®

Alferon N Injection® is the registered trademark for our injectable formulation of natural alpha interferon, which was approved by the FDA for the treatment of certain categories of genital warts. Alferon® is the only natural-source, multi-species alpha interferon currently approved for sale in the U.S. for the intralesional (within lesions) treatment of refractory (resistant to other treatment) or recurring external genital warts in patients 18 years of age or older. Certain types of human papilloma viruses ("HPV") cause genital warts, a sexually transmitted disease ("STD"). The U.S. Centers for Disease Control and Prevention ("CDC") estimates that "*approximately twenty million Americans are currently infected with HPV with another six million becoming newly infected each year. HPV is so common that at least 50% of sexually active men and women get it at some point in their lives.*" Although they do not usually result in death, genital warts commonly recur, causing significant morbidity and entail substantial health care costs.

Interferons are a group of proteins produced and secreted by cells to combat diseases. Researchers have identified four major classes of human interferon: alpha, beta, gamma and omega. Alferon N Injection® contains a multi-species form of alpha interferon. The world-wide market for injectable alpha interferon-based products has experienced rapid growth and various alpha interferon injectable products are approved for many major medical uses worldwide. Alpha interferons are manufactured commercially in three ways: by genetic engineering, by cell culture, and from human white blood cells. All three of these types of alpha interferon are or were approved for commercial sale in the U.S. Our natural alpha interferon is produced from human white blood cells.

The potential advantages of natural alpha interferon over recombinant (synthetic) interferon produced and marketed by other pharmaceutical firms may be based upon their respective molecular compositions. Natural alpha interferon is composed of a family of proteins containing many molecular species of interferon. In contrast, commercial recombinant alpha interferon products each contain only a single species. Researchers have reported that the various species of interferons may have differing antiviral activity depending upon the type of virus. Natural alpha interferon presents a broad complement of species, which we believe may account for its higher activity in laboratory studies. Natural alpha interferon is also glycosylated (partially covered with sugar molecules). Such glycosylation is not present on the currently U.S. marketed recombinant alpha interferons. We believe that the absence of glycosylation may be, in part, responsible for the production of interferon-neutralizing antibodies seen in patients treated with recombinant alpha interferon. Although cell culture-derived interferon is also composed of multiple glycosylated alpha interferon species, the types and relative quantity of these species are different from our natural alpha interferon.

Alferon N Injection® [Interferon alfa-n3 (human leukocyte derived)] is a highly purified, natural-source, glycosylated, multi-species alpha interferon product. There are essentially no neutralizing antibodies observed against Alferon N Injection® to date and the product has a relatively low side-effect profile. The recombinant DNA derived alpha interferon formulations have been reported to have decreased effectiveness after one year, probably due to neutralizing antibody formation.

See “Manufacturing” and “Marketing/Distribution” sections below for more details on the manufacture and marketing/distribution of Alferon N Injection®.

9

HISTORICAL COSTS RELATED TO OUR PRODUCTS

The following table sets forth the costs related to our major products for each of the prior three years. Our aggregate expenses from the time that we first started developing nucleic acid pharmaceutical technology in the mid 1980's through March 2003 were substantially related to the development of Ampligen®, and from that date through the current period were substantially related to Ampligen® and Alferon®.

	(dollars in thousands) Year Ended December 31, 2017			
	Ampligen® NDA	Alferon N® Injection®	Other	Total
Costs and Expenses				
Production costs	$—	$ 1,183	$ —	$1,183
Research and development	3,629	469	—	4,098
General and administrative	4,815	1,757	—	6,572
Total	$8,444	$ 3,409	$ —	$11,853

	(dollars in thousands) Year Ended December 31, 2016			
	Ampligen® NDA	Alferon N® Injection®	Other	Total
Costs and Expenses				
Production costs	$—	$ 1,108	$ —	$1,108
Research and development	4,368	739	—	5,107
General and administrative	5,628	2,053	—	