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Sarepta Therapeutics, Inc. (NasdaqGS: SRPT) — Updating Coverage

Updating Coverage by Reiterating our Strong Buy Rating and increasing our 12-Month Price Target to $170.00

December 30, 2022

Sarepta - The Future Commercial Biotech Discovery Model

Sarepta Therapeutics, Inc. (SRPT) is a commercial-stage biopharmaceutical company focused on RNA targeted therapeutics, gene therapy and other genetic medicine approaches for treating rare neuromuscular diseases.

Current Price	**$127.26**
12 Month Target Price	**$170.00**
12 Month Trading Range	$61.28-$134.08
Market Capitalization (Bil)	$11.173
Shares Outstanding (Mil)	87.8
Avg. Daily Volume	933,301
Debt (Bil)	2.73
Dividend/Yield	N/A
Book Value P/S	4.91
NASDAQ Composite	10,478.09
S&P 500	3,849.28

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The company has been primarily focused on drug candidates for Duchenne Muscular Dystrophy (DMD), a rare, inherited disorder, primarily affecting young males and defined by progressive muscular weakness. It is caused by absence of the protein dystrophin, which connects, strengthens and protects muscle fibers as muscles contract and relax.

DMD is the most common type of muscular dystrophy. 20,000 children are diagnosed with DMD globally each year. Symptoms usually appear between three and five years of age and worsen over time. The disease often occurs in people without a family history of the condition and primarily affects boys, but in rare cases, can affect girls. The condition is universally fatal, and death usually occurs before the age of 30, usually due to respiratory or cardiac failure.

People with DMD progressively lose the ability to perform activities independently and often require a wheelchair by their early teens. As the disease progresses, life-threatening heart and respiratory conditions can occur. Patients typically succumb to the disease in their 20s or 30s.

SRPT has a unique product platform based on fundamental technology to address a tragically fatal disease in boys and young men. The company has both a commercial platform of three DMD therapeutics and a development pipeline of seven clinical stage candidates with four preclinical candidates actively being pursued. SRPT also has earned the good will and support of a vocal and committed parent community which has advocated for accelerated approval of each of the company's products. We therefore are updating our coverage of SRPT by maintaining our Strong Buy rating and increasing our 12-month price target to $170.00.

On November 22, 2022 the FDA accepted the SRPT's Biologics License Application (BLA) seeking accelerated approval of SRP-9001 (delandistrogene moxeparvovec) for the treatment of ambulant individuals with DMD. SRP-9001 has been granted Priority Review by the FDA, with a regulatory action date of May 29, 2023.

Valuation

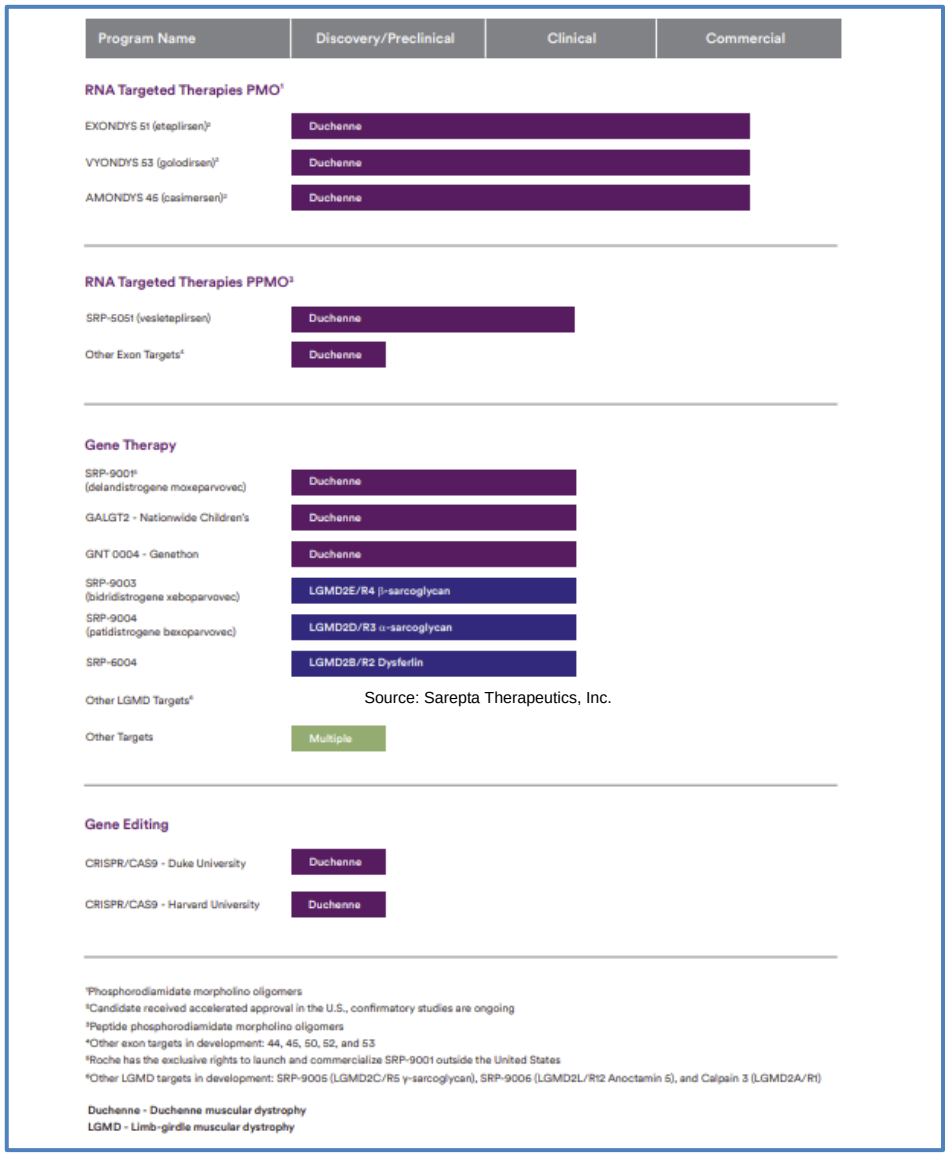
Rating Legend:

Strong Buy – Should be aggressively purchased.
Buy – Should be purchased on market weakness.
Hold – Fairly valued.
Sell – Stock should be sold on market strength.
Sell Short – Should be aggressively sold.
Speculative Buy – For aggressive accounts only.
Core Holding – Essential holding of a long-term account.

SRPT has repeatedly proven itself as an innovator in the life sciences space, both in drug discovery and in its commercial operations. There are many ways to value assets in the healthcare space, but plainspoken models have proven elusive in these tempestuous biotech times. As a result we believe that a multiple based on SRPT's current and our projected revenues makes the most sense for measuring valuation. Our 2023 projected revenue estimates are for $1 Billion and using a multiple of 15 times revenues with a share count of 87.8 million shares we arrive at at 12-month price target of $170.00 per share and continue our Strong Buy Rating.

About DMD

DMD is a rare degenerative neuromuscular disorder causing severe progressive muscle loss due to lack of dystrophin, leading to premature death. Dystrophin is a protein found in muscle cells that is crucial for strengthening and protecting muscle fibers, making up 0.01% of total muscle protein and 5% of the sarcolemma cytoskeletal proteins. DMD is associated with specific errors in the gene that codes for dystrophin. The disease manifests itself in progressive muscle weakness in the lower limbs before spreading to the arms, neck, heart and other areas of the body.



Source: Sarepta Therapeutics, Inc.

Source: Sarepta Web Site December 14, 2022

SRPT has three commercial products, seven clinical stage candidates and five preclinical candidates. There are four categories generally defining the SRPT pipeline: RNA Therapies derived from the company's Phosphorodiamidate morpholino oligomers (PMO) platform, RNA therapies derived from the company's proprietary Peptide phosphorodiamidate morpholino oligomers (PPMO) platform, Gene Therapy and Gene Editing. All programs primarily focus on the DMD indication, with the exception of the Limb-Girdle Muscular Dystrophy (LGMD) candidates.

PMOs, are synthetic molecules modeled after the natural framework of RNA. They are able to bind to specific pre-messenger RNA sequences while remaining highly resistant to degradation. PPMOs, are next-generation PMO-based therapies in development and are specifically designed to increase tissue penetration compared with PMOs. The goal is to learn whether an additional peptide could improve efficacy, reduce dosing and expand the range of treatable diseases.

Commercial-Stage Products:

Each product is administered weekly through intravenous infusions.

EXONDYS 51 (eteplirsen) Injection is SRPT's first commercial product. It received accelerated approval in September 2016 for treatment of DMD in patients who have a confirmed mutation of the gene that is amenable to exon 51 skipping. This product targets the most frequent mutation that causes DMD by producing a shortened but functional substitute of the dystrophin protein. To fulfill FDA post-marketing requirements, the company is conducting two post-market trials – ESSENCE and MISSION.

Recently reported real world data showed that treatment with EXONDYS 51 resulted in statistically significant survival benefits compared to a controlled natural history comparator group of DMD patients. Patients receiving EXONDYS 51 therapy for four-plus years had the greatest benefit.

Hypersensitivity reactions including bronchospasm, chest pain, cough, tachycardia and urticaria, occurred 25% more often in patients who were treated with EXONDYS 51 than those who received placebo. If a hypersensitivity reaction occurs, slowing the infusion or interrupting EXONDYS 51 therapy may be considered. The most common adverse reactions were balance disorder and vomiting. The most common adverse reactions seen in greater than 10% of patients from observational clinical studies were headache, cough, rash and vomiting.

VYONDYS 53 (Golodirsen) Injection is used to treat patients with DMD who have a confirmed mutation in the dystrophin gene that can be treated by skipping exon 53. Eligible patients represent approximately 8% of the DMD patient population. Accelerated approval was granted by the FDA in December 2019 based on an increase in dystrophin production in the skeletal muscles of treated patients. Continued approval is contingent upon verification of a clinical benefit in a confirmatory trial.

A Phase 3 extension study is being sponsored by SRPT to evaluate the long-term safety and tolerability of VYONDYS 53 and AMONDYS 45 in DMD patients who have been treated with these exon-skipping treatments in a clinical trial setting. Boys with mutations amenable to exon 53 skipping will be included in the VYONDYS 53 treatment group, while those with mutations that can benefit from exon 45 skipping will be treated with AMONDYS 45. A total of 260 DMD boys, ages 7 to 23, will be invited to participate in this study.

Patients will receive weekly intravenous infusions of the respective treatment for up to 144 weeks and the number of serious adverse events will be assessed. The estimated completion date is August 2026.

Hypersensitivity reactions, including rash, pyrexia, pruritus, urticaria, dermatitis, and skin exfoliation have occurred in VYONDYS 53 treated patients, some of whom required treatment. If a hypersensitivity reaction occurs, the infusion may be slowed or therapy interrupted.

Kidney toxicity was observed in animals who received VYONDYS 53. Although kidney toxicity was not observed in the clinical studies with VYONDYS 53, the clinical experience with VYONDYS 53 is limited, and kidney toxicity, including potentially fatal glomerulonephritis, has been observed after administration of some antisense oligonucleotides (ASOs).

AMONDYS 45 (Casimersen) injection is an ASO used to treat patients with DMD whose dystrophin gene can be treated by skipping exon 45. AMONDYS 45 received accelerated approval in February 2021 based on an observed increase in dystrophin production in skeletal muscle in treated patients. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials. The ESSENCE trial, a placebo-controlled confirmatory trial to support the AMONDYS 45 approval, is ongoing and expected to conclude in 2024.

Adverse reactions occured in 20% of AMONDYS 45 treated patients and with at least 5% more frequency than patients who received an inactive IV infusion. Adverse reactions included were: upper respiratory tract infection, cough, fever, headache, joint pain, and pain in mouth and throat.

Other adverse reactions that occurred in at least 10% of patients treated with AMONDYS 45 and with at least 5% more frequency than in patients who received an inactive IV infusion were: ear pain, nausea, ear infection, pain after injury, dizziness and light-headedness.

Damage to the kidneys was seen in animals who received AMONDYS 45. Although damage to the kidneys was not seen in clinical studies with AMONDYS 45, potentially fatal kidney damage has occurred with other drugs that work in a similar way. Urine and blood testing before starting treatment, followed by urine testing every month and a blood test every three months to monitor the patient's kidneys before treatment may be recommended.

Clinical-Stage Candidates

SRP-5051 (vesleteplirsen) skips exon 51 of the dystrophin gene. Approximately 30% of DMD patients have the potential to be treated by this drug. SRP-5051 binds to the pre-mRNA of exon 51, resulting in exclusion of this exon during mRNA formation. SRPT's chemistry platform is used to attach a proprietary cell-penetrating peptide to the PMO backbone. The resulting product increases tissue penetration and exon skipping to significantly increase dystrophin production. The PPMO conjugate has the potential to improve efficacy and reduce frequency of patient dosing.

In 2021, the Company announced results from Part A of the MOMENTUM trial, showing that after 12 weeks, 30 mg/kg of SRP-5051 dosed monthly resulted in 18 times the exon skipping and eight times the dystrophin production as eteplirsen, dosed weekly for 24 weeks.

The MOMENTUM study is a Phase 2, multi-arm, ascending dose trial of SRP-5051, infused monthly. This trial will assess dystrophin protein levels in skeletal muscle tissue following SRP-5051 treatment. The trial will enroll up to 60 participants, both ambulant and non-ambulant, between the ages of 7 and 21 at sites in the U.S., Canada, and the European Union. The trial also will assess safety and tolerability.

In June, 2022 the FDA placed a clinical hold on Part B of the MOMENTUM trial of SRP-5051 when reversible hypomagnesemia was identified in patients taking SRP-5051. The hold was removed in September of the same year. Information was provided to the FDA to assess the adequacy of SRPT's risk mitigation and safety monitoring plan. Following discussions with the FDA, and as part of the trial resumption, SRPT will adjust the global trial protocol to include expanded monitoring of urine biomarkers. The protocol for Part B of MOMENTUM now includes magnesium supplementation and monitoring of magnesium levels.

SRP-9001 (delandistrogene moxeparvovec) is an investigational gene transfer therapy intended to deliver SRP-9001 to muscle tissue for the targeted production of functional components of dystrophin. SRP-9001 is a one-time treatment designed to treat the underlying cause of DMD by delivering a functional shortened dystrophin into the muscle tissue.

In December 2019 Roche Holding AG (RHHBY) partnered with SRPT on development of SRP-9001. Under the terms of the agreement, SRPT continues to be responsible for clinical development and manufacturing of SRP-9001. Global clinical development costs are shared equally with RHHBY. SRPT retains domestic commercialization rights and RHHBY has the exclusive right to launch and commercialize SRP-9001 outside the U.S. At closing, RHHBY paid $750 million in cash and $400 million in equity. The agreement provided for up to an additional $1.7 billion in regulatory and sales milestones, plus royalties on net sales.

A BLA for SRP-9001 was submitted for accelerated approval and priority review on September 29, 2022 and is expected to be accepted by the FDA on May 29, 2023. In clinical trials, SRP-9001 demonstrated positive results at multiple time points, including one, two and four years after treatment, in addition to a consistent safety profile.

Sarepta has proposed its fully-enrolled EMBARK Study (SRP-9001-301) as the post-marketing confirmatory study to support accelerated approval. EMBARK is a global, randomized, double-blind, placebo-controlled clinical trial of SRP-9001, which has recruited 126 participants with DMD between the ages of 4 and 7. The primary endpoint for EMBARK is the assessment of the change in the North Star Ambulatory Assessment (NSAA) total score from baseline to week 52 compared to placebo. Results are expected by EOY- 2023.

The company is completing the protocol for ENVISION, a placebo-controlled study evaluating SRP-9001 in nonambulatory patients. Experience with SRP9001 is expected to be applicable to LGMD candidate development and testing.

GALGT2 is currently being developed in a strategic collaboration with Nationwide Children's Hospital through an exclusive license agreement. An open label Phase 1/2a trial with two participants is now underway with an estimated study completion date of October 2023.

GNT-004 is based on an adeno-associated virus (AAV) capsid and an optimized gene (a shortened version of the gene coding for dystrophin). It was developed by Genethon (non-profit), in partnership with the teams from University of London, Royal Holloway and the Institute of Myology in Paris. It is now being jointly developed by Genethon and SRPT.

The first patient in a Phase 1/2/3 trial of GNT-004 was dosed on April 2021. The trial participants are boys aged 6 to 10 suffering from DMD who are still able to walk. Subjects in the experimental group will receive a single intravenous injection of GNT-004 and will be compared to a placebo group for one year. After one year a cross-over is planned to allow all participants to potentially benefit from the treatment. The trial was approved in France and the UK. Submissions are ongoing in the U.S. and Israel.

The measure of efficacy is the change on the NSAA score at one year. The NSAA is a validated 17-item rating scale that is used to measure functional motor abilities in ambulant children.

SRP-9003 (bidridistrogene xeboparvovec) is in development for the treatment of LGMD2E (also known as beta-sarcoglycanopathy and LGMDR4), devastating monogenic neuromuscular diseases caused by a lack of beta-sarcoglycan (beta-SG) proteins. SRP-9003 is a gene therapy that transduces skeletal and cardiac muscle, delivering a gene that codes for the full-length beta-SG protein, the absence of which is the sole cause of the progressive degeneration and a shortened lifespan characterized by the disease.

On March 18, 2021 SRPT announced first look results of expression data from biopsies in a Phase 1-2 study. Results confirmed sustained protein expression in muscle tissue. The study was begun in 2018 and the progress was evaluated two years after a single administration of SRP-9003. This open label, first-in-human study is evaluating a single intravenous infusion of SRP-9003 among children with LGMD2E between the ages of 4 and 15 years with significant symptoms of disease.

The SRP-9003 study has two cohorts, each studying a different doseage. Patients in both cohorts continued to demonstrate stability in their North Star Assessment for Dysferlinopathies (NSAD) total score and improvements on timed function tests. Efficient transduction in skeletal muscle and robust beta-sarcoglycan protein expression were seen in both dose cohorts following infusion with SRP-9003, and significant creatine kinase (CK) reductions were observed.

Since the previous update from this study in October 2020, there no new drug-related safety signals were observed. There were no decreases in platelet counts outside of the normal range and no evidence of clinical complement activation in either dose cohort.

In an exploratory evaluation of all SRP-9003-treated patients compared to a natural history cohort, patients treated with SRP-9003 demonstrated significant improvements in functional outcomes after 24 months. The mean decline in total NSAD score for patients in the natural history cohort was 4.6 points while SRP-9003 treated patients demonstrated a mean improvement of 4.6 points for a clinically meaningful difference of 9.2 points.

On February 25, 2022, SRPT announced that an additional study for SRP-9003 will soon begin. It will use clinical material for LGMD2E to expand its full experience in more ambulatory and nonambulatory patients. It is also SRPT's goal to start a study with commercial process material for SRP-9003 in 2023.

SRP-9004 (patidistrogene bexoparvovec) is a therapeutic treatment for LGMD2D, which is believed to be the most common of the LGMD maladies. LGMD2D causes weakness of muscles

in the hip, shoulder and abdomen. Symptoms often appear before age 10 but, in some cases, do not appear until adulthood. The severity of the disease varies greatly, even within family members. In some cases, symptoms are mild with no effect on lifespan but, in extreme cases, the disease can be fatal and shorten life expectancy. There are no effects on intelligence or mental function at any age.

A Phase 1/2 clinical trial of SRP-9004 was initiated in February 2015 and concluded in March 2019. Results were most recently reported in September 2022. This study assessed the safety and efficacy of SRP-9004 in people with LGMD2D, assigned to one of three patient groups. The first group (1A) consisted of two wheelchair-dependent adults given a single dose of SRP-9004 in one leg. Three ambulatory patients made up groups 1B and 2. Group 1B received the same dose of SRP-9004 given to 1A but in both legs. Group 2 was a high dose group, given a three-fold larger dose of SRP-9004 in both legs.

The therapy was developed at Nationwide Children's Hospital with support from the Muscular Dystrophy Association and licensed to Myonexus Therapeutics, which was acquired by SRPT in 2019.

SRP-6004 is a therapy to treat LGMD2B, which is characterized by the absence of the protein dysferlin. During the SRPT 3Q-2022 conference call, the company announced SRP-6004 will soon begin to be evaluated in a pilot study.

Management

Douglas Ingram, President, Chief Executive Officer and Board Member since 2017. Prior experience includes General Counsel at Allergan until being named President in 2013. When Allergan was acquired by Actavis in 2015, he moved on to Chase Pharmaceuticals, serving as President and CEO before Sarepta. Mr. Douglas received his JD from the University of Arizona and his BS from Arizona State University. He a member of the Relay Therapeutics Board of Directors.

Bilal Arif, Senior Vice President, Strategy and Operations will become SRPT's Chief Technical Operations Officer, effective December 31, 2023.

Ian M. Estepan, Executive Vice President and Chief Financial Officer since December 2020. In January 2015, he joined Sarepta as Head of Investor Relations. Prior to his appointment as CFO, Mr. Estepan served as Sarepta's Chief of Staff, and oversaw the Investor Relations and Corporate Affairs functions, led the development of Sarepta's strategy and was a key architect in securing $2.5 billion in capital to support the company's growth. Mr. Estepan's prior experience includes healthcare sector analyst at Salomon Smith Barney, The Music Booth, LLP, and the Spectra Financial Group, remaining there until joining Sarepta.

Mr. Estepan holds a BA in psychology with a concentration in pre-medicine from Columbia University, where he graduated magna cum laude. He is a member of Cellarity's Board of Directors. In 2022, he was recognized as a Boston Business Journal CFO of the Year.

William F. Ciambrone, Executive Vice President Technical Operations (until December 31, 2022) leads the Company's external manufacturing and CMO management, quality, supply chain operations, manufacturing science and technology, process and analytical development. Prior experience includes increasingly senior roles at Transkaryotic Therapies and Executive Vice President of the Global Technical Operations group at Shire. Mr. Ciambrone also served at North Safety and Healthcare Products and Mallinckrodt Nuclear Medicine.

Mr. Ciambrone earned a bachelor's degree in Biology from St. Anselm College and attended a graduate program in environmental policy at Brown University. He currently serves on the Board of The Mass BIO Education Foundation.

Louise Rodino-Kaplac, PhD., Executive Vice President, Head of R&D and Chief Scientific Officer since November 2021. Dr. Rodino-Kaplac joined Sarepta in June 2018 as executive vice president, chief scientific officer before becoming head of R&D in December 2020. She has led the design of most of Sarepta's late-stage gene therapy candidates, has built and led Sarepta's Genetic Therapies Center of Excellence (GTCOE) in Columbus, Ohio, and has oversight for Sarepta's Gene Editing Innovation Center (GEIC) in Durham, N.C. She is a National Institutes of Health (NIH) Fellow appointee, and is a current Board member of the Alliance for Regenerative Medicine, as well as a member of the American Society for Gene and Cell Therapy, and the American Academy of Neurology. She is former head of the Laboratory for Gene Therapy Research at Nationwide Children's Hospital and co-founded and served as chief scientific officer of Myonexus Therapeutics before it was acquired by Sarepta in 2019.

At Nationwide Children's Hospital, her efforts drove the decade-long research project that led to micro-dystrophin gene therapy. She also is the inventor of five of Sarepta's limb-girdle muscular dystrophy programs. Overall, her work has led to 11 investigational new drug applications.

Dr. Rodino-Kaplac holds a Bachelor of Science degree in biology from King's College and a Ph.D. in molecular genetics from The Ohio State University.

Risks

In addition to risks normally anticipated in a commercial stage biotechnology company, the following are taken from SRPT's 10Q for 3Q-2022.

Commercial Risk: Failure to gain final approval of EXONDYS 51, VYONDYS 53 or AMONDYS 45, which received accelerated approval from the FDA, could result in significant loss of revenue.

Revenue Risk: If reimbursement policies become unfavorable to SRPT, income would be severely diminished.

Competitive Risk: Other companies, both foreign and domestic could become competitors for SRPT's products.

Regulatory Risk: The FDA, EMA or other regulatory agencies may not agree that endpoints or methodologies of SRPT clinical trials provide clinical benefit.

Historical & Future EPS Performance

EPS	2021	2022	2023
Q1	(2.10)A	(1.20)A	(2.12)E
Q2	(1.02)A	(2.65)A	(2.58)E
Q3	(0.60)A	(2.94)A	(2.18)E
Q4	(1.43)A	(1.85)E	(1.99)E
Year	(5.15)A	(11.64)E	(8.87)E
P/E	NM	NM	NM
EPS Growth	NM	NM	NM
FY Rev. (Mil)	612.40A	923.00E	1,000.0E
FY:DEC			

Other Companies Mentioned in this Report
Roche Holding AG (RHHBY)

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	Percentage of Covered Securities	Percentage of Banking Clients
Buy	84%	6.25%
Hold	11%	
Sell	05%	

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