

WILSON THERAPEUTICS AB (PUBL)

INTERIM REPORT JANUARY 1 – JUNE 30, 2017

April 1 - June 30, 2017

- Net sales amounted to SEK 0.0 M (0.0)
- Loss for the period was SEK 54.6 M (loss: 26.9)
- Loss per share, before and after dilution, totaled SEK 2.12 (loss:1.66)
- At June 30, cash and cash equivalents amounted to SEK 308.3 M (435.6)

January 1 - June 30, 2017

- Net sales amounted to SEK 0.0 M (0.0)
- Loss for the period was SEK 86.0 M (loss: 49.2)
- Loss per share, before and after dilution, totaled SEK 3.34 (loss: 5.86)

Significant events during the period January 1 - June 30, 2017

- Extraordinary general meeting was held in January 2017 and resolved to implement the long term incentive program LTIP 2016
- Data from the successfully completed Phase 2 trial were presented at major scientific conferences
- Board strengthened by the appointments of Dr. Birgitte Volck and Dr. Björn Odlander
- The AGM resolved to implement the long term incentive program LTIP 2017
- US Orphan Drug Designation granted for WTX101 for the treatment of ALS

Jonas Hansson, CEO of Wilson Therapeutics.

Financial overview of the Group

Amounts in SEK 000s	Apr–Jun		Jan-	Jan-Dec	
	2017	2016	2017	2016	2016
Net sales	-	-	-	-	-
Operating loss	-45,794	-26,871	-74,236	-49,157	-113,859
Loss for the period	-54,597	-26,891	-86,102	-49,171	-115,175
Loss per share, before/after dilution (SEK)1)	-2.12	-1.66	-3.34	-5.86	-6.84
Equity at the end of the period	342,750	484,383	342,750	484,383	423,458
Cash flow from operating activities	-25,874	-20,143	-56,561	-38,447	-86,148
Cash and cash equivalents at the end of the period	308,272	435,621	308,272	435,621	386,568

¹⁾ Adjusted for share split 1:10 resolved in April 2016.

[&]quot;During the first half of the year, we presented final data from our successful Phase 2 trial at various international medical conferences. In parallel, we have focused on the preparations for the pivotal Phase 3 trial, and approximately 30 clinical sites in the EU, the US and Israel have now been selected for participation in the study. The proposed Phase 3 study protocol, using copper control as primary endpoint, has been submitted to the FDA. We are currently awaiting their feedback and, provided no significant issues arise in the review process, we aim to initiate the trial before the end of 2017."



CEO STATEMENT

Clinical Phase 2 data presented at major conferences

During the first half of the year, we presented final data from our successful Phase 2 trial of WTX101, a novel copper-protein-binding agent, in Wilson Disease at various international medical conferences. As we have previously reported, the primary endpoint was reached, and the data demonstrated that once-daily dosing of WTX101 resulted in a rapid and significant reduction and control of free copper levels, which is key since elevated levels of free copper are the underlying cause of Wilson Disease. Furthermore, the patients' symptoms were ameliorated with significant improvements in neurological status and patient-reported disability. Also, WTX101 was generally well tolerated and no cases of initial drug-induced neurological worsening, a serious and well-recognized side effect of the available chelator therapies for Wilson Disease, were observed in the study.

The results from the Phase 2 study were selected as a late-breaker oral presentation at the Annual Meeting of the European Association for the Study of the Liver (EASL), in Amsterdam in April. This is the largest scientific conference for medical experts in the field of liver diseases in Europe. The positive results, combined with the fact that no new treatments for Wilson Disease have been developed for several decades, led to significant interest in the presentation. Data from the study were also presented in April at the American Academy of Neurology (AAN) Annual Meeting in Boston, and in June at the International Congress of Parkinson's Disease and Movement Disorders in Vancouver.

Orphan drug designation for the treatment of ALS

In June, the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation for WTX101 for the treatment of patients suffering from Amyotrophic Lateral Sclerosis (ALS). ALS is a devastating neurodegenerative disease in which the nerve cells controlling the body's muscles gradually degenerate, leading to general paralysis and respiratory failure. The existing treatment options are limited and the Orphan Drug Designation is an important milestone for us as it recognizes the potential of WTX101 as a possible new treatment for ALS. We are now exploring the possibility of developing WTX101 for the treatment of the subset of ALS patients who suffer from a certain genetic mutation in the copper-dependent enzyme SOD1.

Board strengthened

In May, the Annual General Meeting elected Dr. Birgitte Volck and Dr. Björn Odlander as board members. Dr. Volck is Senior Vice President, Head of R&D Rare Disease at GlaxoSmithKline. Dr. Odlander is Managing Partner and co-founder of HealthCap, a leading European venture capital firm. Both have significant experience from the biotech industry and from development of orphan drugs. Dr. Volck has worked primarily in R&D and medical affairs, and Dr. Odlander is one of the most experienced biotech investors in Europe, so we are of course very excited to welcome them to our board.

Preparations for Phase 3 progressing

In parallel with the analysis of the Phase 2 data, we have focused on the preparations for the pivotal Phase 3 trial, and approximately 30 clinical sites in the EU, the US and Israel have now been selected for participation in the study.

During the period, we have met with the FDA to discuss the trial design. The proposed Phase 3 trial design is a 48-week, randomized, controlled, non-inferiority study evaluating the efficacy and safety of once-daily WTX101 treatment versus standard of care. Approximately 100 Wilson



Disease patients with hepatic and/or neurological symptoms, either treatment naïve or previously treated with an approved Wilson Disease therapy, will be enrolled. Control of free copper levels has been proposed as the primary endpoint for the study. Additional endpoints will include clinical and quality of life related endpoints as well as safety of WTX101. Patients completing the 48-week study will be offered the opportunity to continue treatment with WTX101 in an extension study.

The proposed Phase 3 study protocol has been submitted to the FDA. We are currently awaiting their feedback and, provided no significant issues arise in the review process, we aim to initiate the trial before the end of 2017.

Continued focus on Phase 3 for the remainder of the year

Our continued focus through the rest of the year will be on preparing and initiating our Phase 3 trial. With positive Phase 2 data, a solid balance sheet and an experienced international team, we are well positioned to advance WTX101 into the next development phase.

WTX101 has shown potential to address many of the unmet medical needs in Wilson Disease and, as a result, we are seeing significant support from physicians, as well as the patients and their families, for our work with WTX101. This is a great source of inspiration for all of us at the company as we move closer to the next development phase and take another big step towards reaching our overall goal of improving the lives of patients with rare diseases.

Stockholm August 24, 2017



Jonas Hansson CEO Wilson Therapeutics AB (publ)



PROJECT OVERVIEW

Pipeline

Product	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	Reg.
WTX101	Wilson Disease					
	ALS					

Wilson Disease

Wilson Disease is a genetic disorder of impaired copper transport, caused by loss of function of the ATP7B copper-binding protein, leading to impaired excretion of copper into the bile. This results in accumulation of free copper in the bloodstream, and ultimately in damaging accumulation of copper in the liver, brain and other organs. Copper is an essential trace element that plays a critical role in key physiological cellular processes. Due to its toxic potential, copper is normally tightly bound to copper carrying proteins inside the liver, and excess copper is eliminated from the body via biliary excretion. Untreated Wilson Disease inevitably leads to life-threatening hepatic, neurologic or psychiatric problems, or their various combinations.

Wilson Disease affects approximately one in every 30,000 people worldwide, corresponding to a prevalence of approximately 10,000 patients in the US and 15,000 patients in the EU. The therapies currently being used in Wilson Disease were introduced in the 1950's and 60's and since then there have been no new treatment options developed for patients with this disease.

Treatment goals in Wilson Disease are centered on compensating for impaired copper metabolism, reducing toxic free copper and maintaining normal copper levels to improve the patients' symptoms. The drugs available today are penicillamine and trientine (so called general chelators), and zinc. Chelators bind and reduce the body's copper levels by increasing copper excretion via urine. Zinc reduces dietary uptake of copper in the gut.

These treatments are all associated with significant shortcomings. With current treatments, improving symptoms can take several years and more than a third of patients presenting with neurologic symptoms show no improvement after several years of treatment. Despite being standard of care, many patients receiving chelators develop side effects, some very serious. One of the most serious side effects is that approximately 25% of patients initiated on penicillamine and trientine experience a drug-induced neurological worsening during the initial phase of treatment. Up to 50% of patients who experience neurological worsening never recover to pretreatment level and may be seriously disabled.

Chelators are administered as oral capsules two to four times per day on an empty stomach at least one hour before or two hours after meals, and at least one hour before or after any other pharmaceutical product or milk.



Zinc is recommended as an alternative to chelator therapy for first line treatment of asymptomatic or pre-symptomatic patients. But because of its slow onset of effect (6–12 months), during which time the disease can progress, zinc is generally reserved for maintenance therapy. Zinc has been shown to be less effective than chelating agents. One of the relatively common side effect of zinc is gastric irritation.

Zinc is administered as oral capsules up to five times per day on an empty stomach at least one hour before or two to three hours after meals.

WTX101

WTX101 (bis-choline tetrathiomolybdate) is a first-in-class copper-protein binding agent with a unique mechanism of action, under investigation as a novel therapy for Wilson Disease. WTX101, unlike current treatments for Wilson Disease, enables an alternative copper-protein transport mechanism by rapidly forming copper-protein complexes with high specificity for copper, which quickly de-toxifies copper in both the liver and the blood circulation. WTX101 reduces copper overload by promoting excretion of copper via the bile, the body's natural route for excess copper elimination.

A Phase 2 study evaluating the efficacy and safety of WTX101 in Wilson Disease patients was successfully completed in 2016. In addition, the active ingredient of WTX101, tetrathiomolybdate, has been tested in several previous clinical studies in Wilson Disease patients. The data from these studies suggest that WTX101 can rapidly lower and control toxic free copper levels and improve clinical symptoms in these patients. The data also suggest that WTX101 is generally well tolerated and has the potential for a reduced risk of neurological worsening after initiation of therapy. WTX101 is expected to have a once-daily dosing regimen which may potentially translate into improved compliance in Wilson Disease patients, leading to fewer treatment failures and ultimately improved outcomes as a result. WTX101 has received orphan drug designation for the treatment of Wilson Disease in the US and EU.

In addition, WTX101 has shown potential as a treatment for several other medical conditions including Amyotrophic Lateral Sclerosis (ALS). WTX101 has received US orphan drug designation for the treatment of ALS.

Project status

In November 2016, the Phase 2 study WTX101-201 was concluded. The study was a 24-week open-label Phase 2 study evaluating the efficacy and safety of WTX101 monotherapy in 28 adult newly-diagnosed patients with Wilson Disease, who had received either no prior treatment for Wilson Disease or a standard of care agent for up to two years. All patients who successfully completed the 24-week study period elected to stay on WTX101 in an extension phase of the study.

On April 22, 2017, the final data were presented as a late breaker presentation at the annual EASL liver meeting. The results were also presented at the annual meetings of the American Academy of Neurology (AAN) and of the International Parkinson and Movement Disorder Society. The study met its primary endpoint of copper control (p<0.001). In the ITT population, WTX101 monotherapy reduced mean serum free copper by 72% (p<0.0001) at week 24 compared to baseline. Mean serum free copper levels were reduced below the upper limit of normal after 12 weeks of treatment.

Secondary endpoints included various hepatic and neurological measures. Liver function, as measured by modified Nazer score, remained stable at week 24. Liver status, as measured by



Model for End-Stage Liver Disease (MELD), was also unchanged throughout the study, indicating stabilization. Neurological disability and status were measured as Unified Wilson Disease Rating Scale (UWDRS) part 2 and 3 respectively. Significant neurologic improvements from baseline to week 24 were observed in patients' disability (p<0.001) and neurological status (p<0.0001).

Treatment with WTX101 was generally well tolerated with most reported adverse events being mild (grade 1) to moderate (grade 2). Reversible early liver transaminase elevations were reported in 39% of patients. These elevations were generally mild to moderate, asymptomatic and normalized with dose adjustments. No initial drug-induced neurological worsening upon initiation of WTX101 was observed.

Further detailed data from the WTX101-201 study will be presented at upcoming medical meetings and a pivotal Phase 3 trial is expected to be initiated in the second half of 2017.

ALS

Amyotrophic lateral sclerosis (ALS) is a neurodegenerative disease, in which the nerve cells controlling the body's muscles gradually atrophy, leading to general paralysis and respiratory failure. There are two approved drugs on the market but there is no effective treatment to control the disease. Median survival for an ALS patient is three to five years. About 1.5 to 2.5 per 100,000 people are diagnosed with ALS every year, and about 30,000 people suffer from the disease in the EU and the US. Approximately 7% of the ALS patients have a mutation in a copper dependent enzyme called Superoxide Dismutase 1 (SOD1).

The active ingredient of WTX101, tetrathiomolybdate, has been tested in mice that are genetically modified with a mutant form of human SOD1 and develop ALS. These studies show that tetrathiomolybdate can both delay the onset of disease as well as reduce the symptoms after disease onset in this mouse model.

Project status

Wilson Therapeutics is exploring the possibility of developing WTX101 for the treatment of patients with SOD1-mutated ALS.



SIGNIFICANT EVENTS

Events during the period January 1 – June 30, 2017

Extraordinary general meeting held and resolved to implement the long term incentive program LTIP 2016

An extraordinary general meeting was held on January 18, 2017. The meeting resolved to implement a new long term incentive program (LTIP 2016) for certain senior executives and key employees and to implement a similar performance based long term incentive program (Board LTIP 2016) for certain board members of the company. To ensure delivery of shares under these programs, the general meeting further resolved to issue not more than 392,500 warrants.

Data from the successfully concluded Phase 2 trial of WTX101 were presented at major medical conferences

In April, final data from the company's Phase 2 trial of WTX101 in Wilson Disease were presented as a late breaker presentation at The International Liver Congress™ 2017. The congress is the Annual Meeting of the European Association for the Study of the Liver (EASL), held in Amsterdam, the Netherlands, 19–23 April. The data were presented by Prof. Karl Heinz Weiss, MD, University of Heidelberg. Additional detailed data highlighting the results on neurological parameters were also presented as a poster presentation at the American Academy of Neurology (AAN) Annual Meeting in Boston, MA, 22–28 April, and at the International Congress of Parkinson's Disease and Movement Disorders in Vancouver, BC, 4–8 June. The posters were presented by Danny Bega, MD, Northwestern Memorial Hospital.

Board strengthened by the appointments of Dr. Birgitte Volck and Dr. Björn Odlander

The Annual General Meeting elected Dr. Birgitte Volck and Dr. Björn Odlander as new members of the Board of Directors. Dr. Volck is Senior Vice President, Head of R&D Rare Disease at GlaxoSmithKline in London, UK. Dr. Volck has extensive development experience with orphan drugs and more than 17 years of experience from senior roles in the biotech industry. Dr. Björn Odlander is Managing Partner and co-founder of HealthCap, a leading European venture capital firm. Dr. Odlander has more than 25 years of experience from the biotech industry and he has been involved in a number of companies that have brought novel therapies for rare diseases to market.

The AGM resolved to implement the long term incentive program LTIP 2017

The Annual General Meeting resolved to implement a new long term incentive program (LTIP 2017) for certain senior executives and key employees in the Wilson Therapeutics group and to implement a similar performance based long term incentive program (Board LTIP 2017) for certain board members of the company. To ensure delivery of shares under these programs, the general meeting further resolved to issue not more than 155,100 warrants.

US Orphan Drug Designation granted for WTX101 for the treatment of ALS

In June, the U.S. Food and Drug Administration (FDA) granted the company Orphan Drug Designation for WTX101 for the treatment of patients suffering from Amyotrophic Lateral Sclerosis (ALS). ALS is a devastating neurodegenerative disease, in which the nerve cells controlling the body's muscles gradually degenerate, leading to general paralysis and respiratory failure. Median survival for an ALS patient is three to five years. Orphan Drug Designation provides the sponsor certain benefits and incentives, including a period of marketing exclusivity if regulatory approval of the drug is ultimately received for the designated indication, potential tax credits for certain activities, eligibility for orphan drug grants, and the waiver of certain administrative fees.



Events after the end of the report period

No significant events have occurred after the end of the report period.



FINANCIAL INFORMATION

Sales and earnings for the second quarter of 2017

Sales amounted to SEK 0.0 M (0.0) and the operating result deteriorated to a loss of SEK 45.8 M (loss: 26.9). The loss for the second quarter was SEK 54.6 M (loss: 26.9). The cost increase was due mainly to higher costs related to the upcoming Phase 3 clinical trial and costs for the company's long term incentive programs.

Sales and earnings for the first six months of 2017

Revenue

Sales amounted to SEK 0.0 M (0.0) during the period. The company is not expected to generate any revenue until its products are launched onto the market.

Sales and administrative expenses

During the period, sales and administrative expenses increased to SEK 24.7 M (20.8). The increase was largely a result of increased costs for commercial planning for the future launch of WTX101 amounting to SEK 4.3 M (0.0) and increased recorded non-cash costs for the company's employee long term incentive programs, amounting to SEK 9.8 M (6.7).

Research and development expenses

During the period, research and development expenditure increased to SEK 50.4 M (28.2). The increase was mainly due to increased costs for preparations for the upcoming Phase 3 clinical trial including clinical costs, CMC related costs and regulatory expenses. Non-cash costs for the company's long term incentive programs increased to SEK 5.9 M (3.0).

Loss for the first six months totaled SEK 86.0 M (loss: 49.2), resulting in a loss per share, before and after dilution, of SEK 3.34 (loss: 5.86).

Liquidity and financing

In the first six months of the year, cash flow from operating activities increased to a negative SEK 56.6 M (neg: 38.4), largely due to increased costs for the clinical trial activities. Cash flow from investing activities was a negative SEK 16.2 M (0.0) mainly due to a reclassification of bank holdings into financial fixed assets as the holdings are held as collateral for the company's currency hedging instruments.

Cash flow from financing activities amounted to SEK 0.0. M (442.6), as new share issues were executed in the first six months of 2016 but not in 2017.

Cash flow for the period was a negative SEK 72.8 M (pos: 404.2). At June 30, 2017, cash and cash equivalents amounted to SEK 308.3 M, compared with SEK 435.6 M at June 30, 2016.

Share-based incentive programs

The purpose of share-based incentive programs is to promote the company's long-term interests by motivating and rewarding the company's senior executives and other employees.

Wilson Therapeutics has implemented an employee stock option program comprising employees, certain board members and certain consultants. During the period, no stock options



have been granted and no stock options have been exercised. At June 30, the number of granted stock options amounted to 1,765,000 (1,765,000).

In February 2017, a long-term incentive program for certain senior executives and key employees (LTIP 2016) was implemented and a similar program was implemented for certain board members (Board LTIP 2016). In June, as resolved by the Annual General Meeting, another long-term incentive program for certain senior executives and key employees (LTIP 2017) was implemented and a similar program was implemented for certain board members (Board LTIP 2017). All these programs are performance-based share award programs entitling the holder to one common share in Wilson Therapeutics per share award after three years. In the programs for employees the share awards will vest provided certain operational targets relating to share price development are met. In the programs for board members the share awards will vest provided certain targets only relating to the share price are met. If a takeover occurs during the three-year vesting period, a proportion of the share awards will vest to the extent the performance conditions are met at that point in time. In total, share awards entitling to 547,600 common shares were granted, out of which 487,500 pertain to the program for the employees and 60,100 to the program for board members. Senior executives were granted 72% of the share awards.

The average value per granted share award was SEK 39.36. The valuation was performed using a discounted value of the outcome where the share price development was simulated using Monte Carlo simulation for the share price related targets, and the probabilities were estimated for the outcome of the operational targets. The valuation assumed no future dividends. 35,000 share awards have lapsed and no share awards have been converted into common shares during the period and at June 30 the total number of outstanding share awards granted amounted to 512,600 (0).

Costs for these programs of SEK 15.7 M (9.6) were charged to earnings for the quarter but have no cash impact. The company's other provisions amounting to SEK 21.5 M (6.8) are provisions for social contributions related to the incentive programs.

Investments

Fixed assets mainly consist of intellectual property rights. During the first six months of the year investments in intangible fixed assets amounted to SEK 0.0 M (0.0), and investments in tangible fixed assets to SEK 0.0 M (0.0). Investments in financial fixed assets amounted to SEK 15.3 M (0,0) and were related to bank holdings held as collateral for the company's currency options.

Employees

At June 30, 2017, Wilson Therapeutics had 14 (9) employees. The average number of employees was 14 (8) in the first six months of 2017.

Parent Company

Since the operations of the Parent Company are consistent with those of the Group in all material respects, the comments for the Group are also largely relevant for the Parent Company.



CONDENSED CONSOLIDATED STATEMENT OF COMPREHENSIVE INCOME

Amounts in SEK 000s	Apr-	Jun	Jan-	Jun	Jan-Dec
	2017	2016	2017	2016	2016
Net sales	-	-	-	-	-
Gross profit	-	-	-	-	-
Sales and administrative expenses	-16,100	-11,726	-24,650	-20,835	-42,208
Research and development expenses	-30,166	-15,046	-50,387	-28,201	-70,851
Other operating revenue	472	-	801	20	20
Other operating expenses	-	-99	-	-141	-820
Operating loss	-45,794	-26,871	-74,236	-49,157	-113,859
Net financial items	-8,431	0	-11,354	1	-1,245
Loss before tax	-54,225	-26,871	-85,590	-49,156	-115,104
Tax	-372	-20	-403	-15	-71
Loss for the period ¹⁾	-54,597	-26,891	-85,993	-49,171	-115,175
Other comprehensive income					
Items that will be reclassified to profit or loss					
Translation difference for the period	-78	43	-109	18	123
Other comprehensive income after tax for the period	-78	43	109	18	122
Comprehensive loss for the period ¹⁾	-54,675	-26,848	-86,102	-49,153	-115,052
Loss per share, before/after dilution (SEK) ²⁾	-2.12	-1.66	-3.34	-5.86	-6.84

^{1) 100%} attributable to Parent Company shareholders.

²⁾ Adjusted for share split 1:10 resolved in April 2016. Earnings per share calculated as earnings per common share, where the result is adjusted for the right of preference shareholders to receive a dividend for the period. In 2016, all preference shares were converted into common shares in connection with the stock market listing.



CONDENSED CONSOLIDATED STATEMENT OF FINANCIAL POSITION

Amounts in SEK 000s	JUN 30, 2017	JUN 30, 2017	DEC 31, 2016
ASSETS			
Intangible fixed assets	64,632	64,632	64,632
Tangible fixed assets	45	6	55
Financial fixed assets	15,410	54	151
Total fixed assets	80,087	64,692	64,838
Current reasingles	4.040	0.704	0.400
Current receivables	4,810	2,704	2,480
Cash and cash equivalents	308,272	435,621	386,568
Total current assets	313,082	438,325	389,048
TOTAL ASSETS	393,169	503,017	453,886
EQUITY AND LIABILITIES			
Equity attributable to shareholders of the parent company	342,750	484,383	423,458
Other provisions	21,504	6,774	11,167
Other liabilities	2,531	-	-
Total non-current liabilities	24,035	6,774	11,167
Accounts payable	15,658	5,964	8,155
Other current liabilities	10,726	5,896	11,106
Total current liabilities	26,384	11,860	19,261
Total Current Habilities	20,304	11,000	13,201
TOTAL EQUITY AND LIABILITIES	393,169	503,017	453,886



CONDENSED CONSOLIDATED STATEMENT OF CHANGES IN EQUITY

Amounts in SEK 000s	Apr-	Jun	Jan-	Jun	Jan-Dec
	2017	2016	2017	2016	2016
Opening balance	394,925	105,024	423,458	84,562	84,562
Comprehensive loss for the period	-54,675	-26,848	-86,102	-49,153	-115,052
New share issue	-	435,617	-	475,617	475,617
Costs attributable to new share issue	-	-32,876	-	-32,976	-32,976
Employee stock option program	2,500	3,466	5,394	6,333	11,307
Total transactions with owners	2,500	406,207	5,394	448,974	453,948
Closing balance	342,750	484,383	342,750	484,383	423,458

CONDENSED CONSOLIDATED STATEMENT OF CASH FLOW

Amounts in SEK 000s	Apr-	Jun	Jan-	Jun	Jan-Dec
	2017	2016	2017	2016	2016
Operating loss	-45,794	-26,871	-74,236	-49,157	-113,859
Adjustment for non-cash items1)	12,149	5,932	15,742	9,667	19,047
Interest received	181	1	313	1	3
Interest paid	-3	-6	-3	-6	-9
Tax paid	-430	77	-436	-71	-68
Cash flow from operating activities before changes in working capital	-33,897	-20,867	-58,620	-39,566	-94,886
Cash flow from changes in working capital	8,023	724	2,059	1,119	8,738
Cash flow from operating activities	-25,874	-20,143	-56,561	-38,447	-86,148
Cash flow from investing activities	-1,287	-	-16,231	-	-160
Cash flow from financing activities	-	402,742	-	442,641	442,641
Cash flow for the period	-27,161	382,599	-72,792	404,194	356,333
Cash and cash equivalents at the beginning of the period	339,268	52,988	386,568	31,404	31,404
Exchange-rate difference in cash and cash equivalents	-3,835	34	-5,504	23	-1,169
Cash and cash equivalents at the end of the period	308,272	435,621	308,272	435,621	386,568

¹⁾ Pertains mainly to costs of share based incentive programs including social contributions.



CONDENSED PARENT COMPANY PROFIT AND LOSS STATEMENT

Amounts in SEK 000s	Apr–	Jun	Jan-	Jan-Dec	
	2017	2016	2017	2016	2016
Net sales	-	-	-	-	-
Gross profit	-	-	-	-	-
Sales and administrative expenses	-16,070	-11,719	-24,555	-20,766	-42,116
Research and development expenditure	-30,332	-15,067	-50,825	-28,259	-71,489
Other operating revenue	472	-	801	-	0
Other operating expenses	-	-99	-	-141	-820
Operating loss	-45,930	-26,885	-74,579	-49,166	-114,425
Profit/loss from financial items	-8,431	1	-11,354	1	-1,245
Loss after financial items	-54,361	-26,884	-85,933	-49,165	-115,670
Tax	-	-	-	-	-
Loss for the period	-54,361	-26,884	-85,933	-49,165	-115,670

CONDENSED PARENT COMPANY STATEMENT OF COMPREHENSIVE INCOME

Amounts in SEK 000s	Apr–Jun		Jan-	Jan-Dec	
	2017	2016	2017	2016	2016
Loss for the period	-54,361	-26,884	-85,933	-49,165	-115,670
Other comprehensive income	-	-	-	-	-
Comprehensive loss for the period	-54,361	-26,884	-85,933	-49,165	-115,670



CONDENSED PARENT COMPANY BALANCE SHEET

Amounts in SEK 000s	JUN 30, 2017	JUN 30, 2016	DEC 31, 2016
ASSETS			
Intangible fixed assets	32,360	32,360	32,360
Tangible fixed assets	45	6	55
Financial fixed assets	47,841	32,485	32,582
Total fixed assets	80,246	64,851	64,997
Current receivables	4,860	2,721	2,560
Cash and cash equivalents	306,627	434,458	385,498
Total current assets	311,487	434,179	388,058
TOTAL ASSETS	391,733	502,030	453,055
EQUITY AND LIABILITIES			
Restricted shareholders' equity	2,858	2,858	2,858
Unrestricted shareholders' equity	338,533	480,602	419,071
Total Equity	341,391	483,460	421,929
Other provisions	21,504	6,774	11,167
Other non-current liabilities	2,531	-	-
Accounts payable	15,576	5,847	8,147
Other current liabilities	10,731	5,949	11,812
Total current liabilities	26,307	11,796	19,959
TOTAL EQUITY AND LIABILITIES	391,733	502,030	453,055



NOTES

Note 1 Accounting policies in accordance with IFRS

This interim report for the Group has been prepared in accordance with IAS 34 Interim Financial Reporting and the applicable provisions of the Swedish Annual Accounts Act. The interim report for the Parent Company has been prepared in accordance with the Swedish Annual Accounts Act and RFR2 Reporting for legal entities. The same accounting policies and measurement criteria have been applied for the Group and the Parent Company as in the 2016 Annual Report. New or revised IFRS requirements introduced in 2017 have not affected Wilson Therapeutics during the period.

The group presents the alternative key ratio operating profit/loss as it is deemed useful as a complement to other key ratios to understand the group's financial reports. The group does not present any other alternative key ratios as no such key ratios are deemed necessary to understand the group activities.

Note 2 Risks and uncertainties in the Group and Parent Company

Operational risks

Wilson Therapeutics' main operations are research and development of pharmaceutical products, which is to a large extent both a high-risk and capital-intensive field. Most of the initiated projects will never reach market registration due to the risk that the drug does not show sufficient efficacy or has a problematic side effect profile and, after launch, products may have their regulatory licenses withdrawn as side effects emerge. If competing pharmaceutical products capture market share or competing research projects achieve better efficacy and reach the market more rapidly, the future value of the product portfolio could be lower than expected. The operations are also impacted by decisions from public authorities, such as approvals and price changes. There is an ongoing political debate, particularly in the US, regarding perceived excessive orphan drug pricing. There is a risk that new regulations will have a negative impact on orphan drug prices in the future. There are also risks concerning the manufacturing of pharmaceutical products. The chosen manufacturer may become unable to manufacture sufficient quantities and/or quality or may lose necessary manufacturing approvals.

Financial risk management

Wilson Therapeutics' financial policy governing the management of financial risks has been designed by the Board of Directors and represents a framework of guidelines and rules in the form of risk mandates and limits for financial activities. Wilson Therapeutics is primarily affected by foreign-exchange risk. A considerable portion of the company's costs is denominated in USD and EUR. A portion of the company's cash, amounting to the expected need for the coming 6-month period, is held in USD. The company also holds currency options to secure expected further USD needs amounting to USD 12.2 M. The financial policy is updated at least once annually.

For a more detailed description of risks and uncertainties, refer to Note 18 in the 2016 Annual Report.

Note 3 Financial instruments

Wilson Therapeutics' financial assets and liabilities comprise cash and cash equivalents, accrued expenses and accounts payable and currency options. Except for currency options, the fair values of all financial instruments with short maturities are approximately equal to their



carrying amounts. The currency options have been classified as valued at fair value over the profit and loss statement and have been valued using level 2 inputs according to the IFRS 13 hierarchy. The book value of the currency options was a negative SEK 5.3 M (0.0) as of the balance sheet day. Wilson Therapeutics has not applied net accounting to any financial assets or liabilities, and has no agreements that permit offsetting.

Note 4 Cash and cash equivalents

The company's cash and cash equivalents consists of cash deposits at the company's bank.

Note 5 Change in number of shares

	Apr–Jun		Jan-	Jan-Dec	
	2017	2016	2017	2016	2016
Total number of shares, opening balance	25 720 248	1 683 010	25 720 248	1 531 926	1 531 926
Shares added through share split 1:10	-	15 147 090	-	15 147 090	15 147 090
Shares added through new share issues	-	8 890 148	-	9 041 232	9 041 232
Total number of shares, closing balance	25 720 248	25 720 248	25 720 248	25 720 248	25 720 248

Note 6 Related-party transactions

During the period a share based incentive program has been implemented for senior executives. No other transactions have taken place between the company and its related parties that could materially affect the company's position and earnings for the period.

Note 7 Estimates and judgments

Preparation of the interim report requires management to make estimates that affect the reported amounts of assets, liabilities, revenues and expenses. Actual outcomes may deviate from these estimates. The key sources of estimation uncertainty are the same as those outlined in Note 2 of the 2016 Annual Report.

Review

This interim report has not been reviewed by the company's auditors.



The Board of Directors and CEO declare that the undersigned interim report provides a fair overview of the parent company's and Group's operations, their financial position and performance, and describes material risks and uncertainties facing the parent company and other companies in the Group.

Stockholm August 24, 2017

Andrew Kay
Chairman of the Board

Dina Chaya Member of the Board Genghis Lloyd-Harris

Member of the Board

Bali Muralidhar

Member of the Board

Björn Odlander Member of the Board Hans Schikan

Member of the Board

Birgitte Volck

Member of the Board

Jonas Hansson *CEO*

The information in the interim report is information that Wilson Therapeutics is obliged to make public pursuant to the EU Market Abuse Regulation and the Securities Markets Act. The information was submitted for publication, through the agency of the contact person set out above, at 08.00 CET on August 24, 2017.



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Financial calendar

Interim report for the third quarter 2017 Year-end report 2017 Interim report for the first quarter 2017 Annual General Meeting 2018 November 23, 2017 February 21, 2018 May 16, 2018 May 16, 2018

About Wilson Therapeutics

Wilson Therapeutics is a biopharmaceutical company, based in Stockholm, Sweden, that develops novel therapies for patients with rare diseases. Wilson Therapeutics' lead product, WTX101, is in development as a novel treatment for Wilson Disease. A Phase 2 clinical study has been successfully completed and preparations for a pivotal Phase 3 study are ongoing. Wilson Therapeutics is listed in the Mid Cap segment on Nasdaq Stockholm with the stock ticker WTX.

Visit <u>www.wilsontherapeutics.com</u> for more information.