February 27, 2014

The Untold Secret that Questcor has been Covering Up:

Acthar Faces Severe Risk of Being Pulled off the Market by the FDA

A Citron Research Exclusive

Citron Research is very aware of the grave nature of the above headline, and the impact of information disclosed in this story. We understand clearly that there are consequences for making statements that are false or based upon incorrect assumptions. We have been exposing fraud for 13 years and have never hidden behind a guise of anonymity. We have always invited our work to be subject to legal scrutiny. That being said, we ask all Questcor shareholders – long and short – to take a big breath, sit down, and read. **Citron publishes what we believe to be our most detailed and explosive report to date on Acthar, and Questcor(NASDAQ:QCOR).**

It is no secret that Acthar is a controversial drug, marketed by Questcor, a highly controversial company. Its hefty price tag, aggressive marketing strategy, and secretive management have landed it in the crosshairs of multiple government agency investigations, including two U.S. Attorneys General, plus the SEC. But this might be the least of their problems. What Citron reveals here will now change the game on Questcor.

Please read the first 5 pages for proper background – you'll understand the route by which the truth came to light.

What Led us to this Study?

For those familiar with the background on Acthar, management consistently tells a story about the "secret sauce" inside the drug that makes it unique. Here is a vintage excerpt in CEO Don Bailey own words:

"I'd like to go through the **barriers to entry** because this is the key question most investors have with respect to the longevity of this unusual asset.

The first barrier to entry is the formulation. Acthar is a biologic. Acthar is an extraction of porcine pituitaries. It's an **undisclosed composition**, so that's a trade secret. The manufacturing process is also a trade secret. It's complex, it's unique, and we own all elements of the manufacturing process. We have exclusive worldwide rights to Acthar, so we own it lock, stock and barrel. We have no partners. The composition of Acthar that comes out of the manufacturing process is tied to the process, so if you don't know the process you can't figure out what's actually in Acthar. Acthar is technically a polypeptide, but there are **probably multiple active ingredients** and there are multiple peptides within Acthar, and **they're undisclosed**."

-- Questcor Investor presentation Aug 22, 2011, filed as an 8-K

http://www.sec.gov/Archives/edgar/data/891288/000119312511225705/dex991.htm

The company's claim of a very difficult manufacturing process and unknown ingredients **supplementing** highly purified ACTH has always been their competitive moat. Management claims this makes Acthar a unique drug for multiple difficult-to-treat diseases, and not possible to compete with.

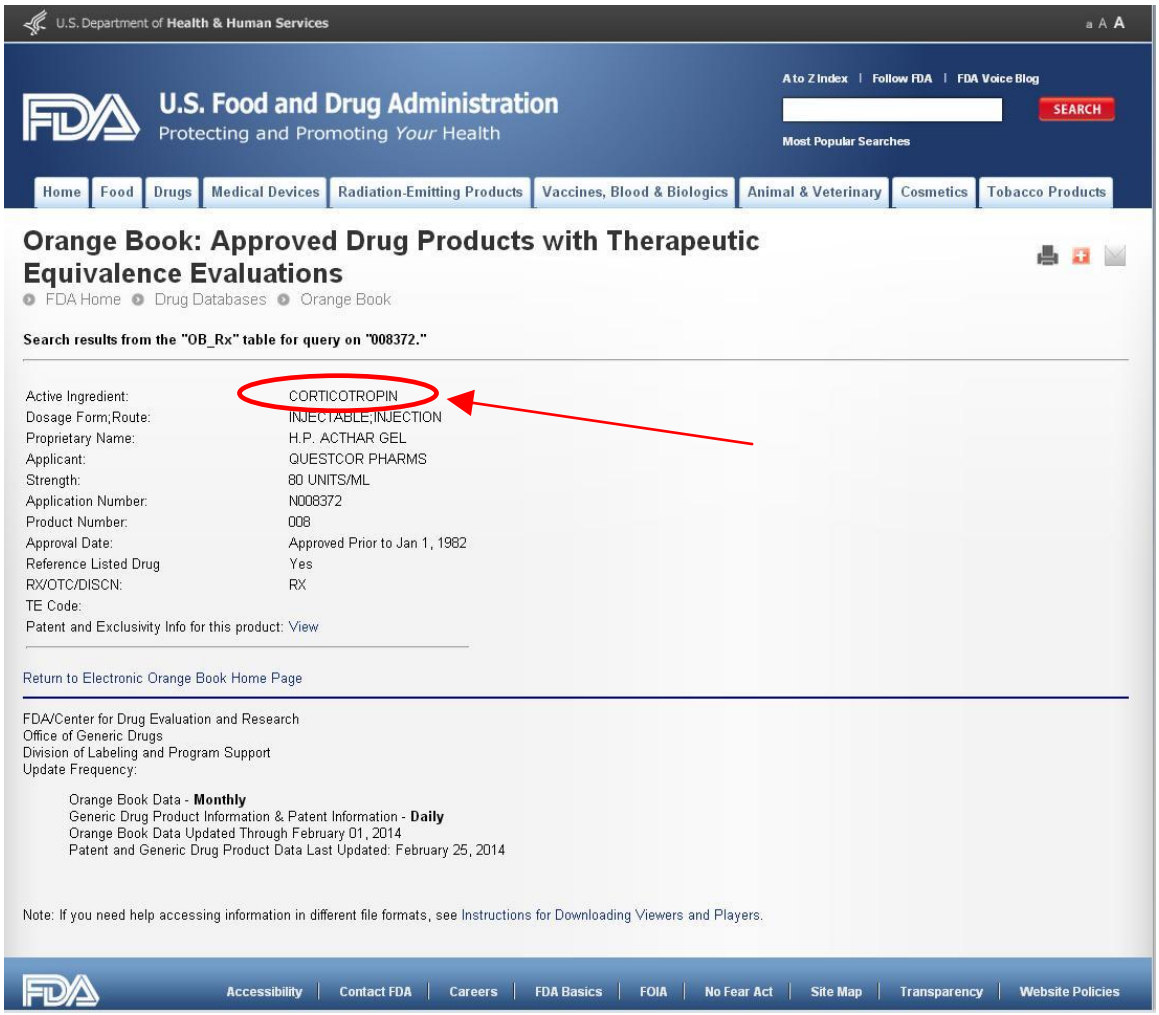
Until now, it has been Citron's strong opinion that evaluating the above statement was pivotal to assessing the barrier to entry for any competitor to Acthar – and therefore, to determine whether the value of Questcor's stock was sustainable.

However, **ALL OF THIS RHETORIC IS COMPLETELY FALSE.** Based on previously undisclosed independent laboratory findings, the above is a blatant misrepresentation to investors and patients alike. That is the essence of today's report.

It is critically important for every Questcor investor, long or short, to understand fully the claims implied by CEO Bailey's statement above.

Acthar's label (revised in 2012), by which it gains grandfathered permission from the FDA to sell Acthar in the United States, states it has one active ingredient: **Corticotropin, or purified ACTH**.

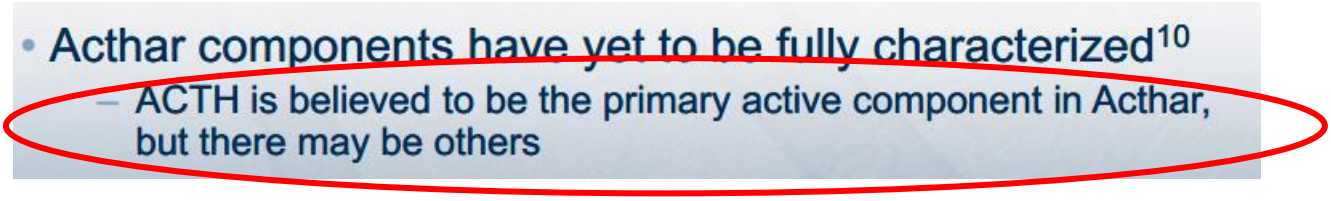
Despite the drug's explicit and unambiguous label, CEO Bailey claims there is "something else" in the vial. Can he blame anyone for trying to find out what it really is? Read on.



http://www.accessdata.fda.gov/scripts/cder/ob/docs/obdetail.cfm?Appl_No=008372&TABLE1=OB_Rx

There is no debate about what Acthar Gel is **supposed to be**. According to its label, which must be carefully approved by the FDA, as specified in the FDA Orange Book, and backed up by Good Manufacturing Practice standards, Acthar is **labeled as**, and **supposed to be corticotropin -- the hormone ACTH**.

In recent years, the company has engaged in an intense word-crafting exercise, suggesting that something else is active beside the ACTH in the drug, which creates a barrier to entry.



JP Morgan Healthcare Conference, Jan 16, 2014

http://files.shareholder.com/downloads/ABEA-2S424C/2977702191x0x718107/7280B20E-3644-434C-AB6F-5F5ACA4FEC6B/QCOR_JPM_v8_Final_for_Handout.pdf

This is a truly intriguing statement. First of all, they misquote their own label (footnote #10), which actually states: **"The pharmacokinetics of H. P. Acthar Gel have not been adequately characterized." Not the components. The pharmacokinetics.**

Next, consider the word "believed". Questcor has now sold Acthar for over seven years, with a current run rate approaching a billion dollars per year, and still they have no idea what's inside the vial, except for what they **"believe"?** How sustainable is that?

For the past few years, we have been assuming that Questcor was invoking this mystical language to create the appearance of a "competitive moat" – the "secret sauce" claim – that it is the presence of other additional biologically active components that make Acthar the special medicine they claim it to be.

But today, we have new information that changes everything.

Concerned investors, trying to understand and evaluate CEO Bailey's statements above, have undertaken a multi-month project, involving biological laboratory testing of Acthar, to find out what Questcor has never disclosed about the product. We all knew what the main active ingredient of Acthar was supposed to be … but what could be learned about the "1%"?

Instead, what was found is beyond appalling.

The state-of-the-art lab results – which have been confirmed at a second laboratory – decisively conclude that in our tests of production Acthar released to patients, from at least **two** separate batches -- nearly all of what is being detected in Acthar is degraded remnants of what **used to be** ACTH. There is almost **no detectable ACTH** in any of the Acthar we tested.

That's right, a world-class laboratory detected little to no ACTH in Acthar.

Background on the lab that conducted the study:

Citron is redacting the identity of the lab to the investing public because the testing is ongoing. While we are confident enough in the consistency of the findings thus far to justify publishing them, we are currently analyzing even more batches of Acthar, and seeking answers to further questions about its composition.

Meanwhile, we all know that Questcor is a litigious company. When investors attempted to attend a charity function for the Chronic Disease Fund, an organization purportedly strictly independent of Questcor, Questcor lawyers sent threatening letters to them. We do not want Questcor to attempt to obstruct the further testing of Acthar currently underway. However, we warrant to the investing public that the labs have top-flight credentials, perform work for many Fortune 500 companies, and have ample facilities for performing this type of work, and in particular, FDA cGMP credentials and experience. We will WILLINGLY share the name of the lab and all test results with ANY government agency that contacts us. We are confident enough in the protocols used to defend this work before any regulatory body.

We publish today significant excerpts of the voluminous findings as delivered to the FDA. Needless to say Citron is neither a biochemical engineer nor an FDA legal expert, so we will not opine on those matters. We will just quote from the sources themselves.

What the FDA Has Just Been Notified Of

The results of these studies, with all the supporting documentation, were summarized and delivered to the FDA in early December 2013 by a highly credentialed attorney at one of Washington D.C.'s five top firms, who formerly served as a top legal enforcement official at the FDA himself.

While not publishing the entire 31 page document as submitted to the FDA, in order to protect the name of the attorney and the firm, Citron publishes for the investing public the most relevant excerpts. For anyone seeking a full copy of this entire submission, including over 270 pages of supporting lab reports, and literally hundreds of liquid chromatography and mass spectrometer measurements in graphic formats, we suggest an FOIA request to FDA. **Meanwhile, Citron will supply this documentation in full to any interested government regulatory agency.**

Here are the key excerpts with the name of the lab redacted. Investors do not need to see the price increase story rehashed again, so we have simply skipped that part. Without further editorial comment, this is what the professionals say.

FDA "Received" date: December 13, 2013
Attn: [REDACTED]
United States Food and Drug Administration
Center for Drug Evaluation and Research
Office of Compliance
10903 New Hampshire Ave
Silver Spring, MD 20993

RE: NDA No. 022432, H.P. Acthar Gel

We write to bring to your attention serious concerns we have with H. P. Acthar Gel® (repository corticotropin injection) ("Acthar Gel"), the subject of New Drug Application No. 022432, sponsored by Questcor Pharmaceuticals, Inc. ("Questcor").

(Page 1)

Herein we report our data and analysis from [*Lab Name*], a [*Lab City*] -based contract laboratory that specializes in biopharmaceutical product characterization, analytical testing, and complex biologics characterization services for the pharmaceutical and medical device industries. **[*Lab Name*] has determined that vials of Acthar Gel it examined contain little or none of the active pharmaceutical ingredient ("API") corticotropin.** Instead, [*Lab Name*] has found that the vials contain a degraded derivative of corticotropin that is known to lack the same potency as corticotropin.

(Page 1)

Based on the results of [*Lab Name*] qualitative and quantitative analyses, it is clear that the Acthar Gel [*Lab Name*] examined is not what Questcor purports it to be. While [*Lab Name*] analyses do not demonstrate why corticotropin is absent from the Acthar Gel -- perhaps because Questcor's formulation is inherently unstable and degrades quickly; or because the manufacturing process is poorly controlled or flawed and causes an unwanted chemical reaction; or because the starting material was simply inadequate -- **[*Lab Name*]'s findings raise important regulatory concerns about the identity, purity and stability of Questcor's drug product that warrant an FDA investigation**.

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As explained further below, Questcor itself admits that it does not know what active ingredient is present in Acthar Gel. Because it appears not to be corticotropin, the sole named active ingredient that FDA reviewed and approved, Acthar Gel is by definition an unapproved new drug. And it follows that neither Questcor, nor any other sponsor, has ever presented data on this new drug to FDA concerning the safety or efficacy for the listed indications, including infantile spasms**.**

The absence of corticotropin in Acthar Gel presents significant health and safety concerns.

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When properly dosed, corticotropin is believed to be an effective treatment for infantile spasms, and was used off-label to treat the syndrome for decades before Questcor secured Acthar Gel's approval for the indication. Patients treated with Acthar Gel, however, may not be getting the proper therapeutic benefit if, as [*Lab Name*] 's data shows, the drug contains little, if any, non-degraded corticotropin.

To protect infants and numerous other patients treated with Acthar Gel that is devoid of corticotropin, we strongly urge FDA to examine Questcor's manufacturing and quality control practices immediately, to determine the extent and cause of the problem, and if necessary, to initiate an appropriate regulatory response to ensure patient safety.

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Additionally, Questcor now admits that the Acthar Gel it markets may have additional active ingredients besides corticotropin. Describing Acthar Gel and its mechanism, Mr. Young told investors:

> "From those hundreds and hundreds of peptides, we're trying to extract just ACTH. But of course if you think about it, if I have this mixture and I'm trying to extract only one molecule, that's very unlikely. Instead what you're doing, you're extracting ACTH plus other peptides that are now in that mixture … So the question we had in 2008 and 2009 is how many active peptides are really in Acthar, and do they have any meaning? Are they clinically significant? So we've been working on the chemistry trying to identify the peptides. We've been working on the pharmacology, trying to determine which ones are active, which ones are not active. And what we found are that there are multiple peptides in Acthar Gel. These multiple peptides have multiple pharmacological properties. They're not exactly the same. They're not qualitatively exactly the same and they're not quantitatively exactly the same."

These statements suggest that the drug product Questcor markets today is comprised of active ingredients that were not identified in the studies it submitted to FDA to obtain approval for the treatment of infantile spasms. **Furthermore [*Lab Name*]'s data suggests that the active ingredient FDA approved, corticotropin, is present in at most only trace amounts.**

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Questcor's CEO Don Bailey has also made statements to investors asserting that the active ingredient in its Acthar Gel is not simply corticotropin:

> "It's an undisclosed composition, so that's a trade secret. The manufacturing process is also a trade secret. It's complex, it's unique, and we own all elements of the manufacturing process. We have exclusive worldwide rights to Acthar, so we own it lock, stock and barrel. We have no partners. The composition of Acthar that comes out of the manufacturing process is tied to the process, so if you don't know the process you can't figure out what's actually in Acthar. Acthar is technically a polypeptide, but there are probably multiple active ingredients and there are multiple peptides within Acthar, and they're undisclosed."

Even without [*Lab Name*] 's recent data finding no corticotropin in tested vials of Acthar Gel, Questcor's own comments as to changes in the manufacturing process and the likely presence of other active peptides merit review and investigation. FDA, of course, requires that an NDA include, among other things, a full list of the components of a drug and statement of its composition. Questcor, however, claims that the 60-year old drug it is now marketing contains "multiple active ingredients" that were not reviewed and evaluated by FDA. **[*Lab Name*] 's testing confirms that claim, at least to the extent it shows that the drug product Questcor markets appears to be different from the product approved by the FDA.**

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2. Qualitative and Quantitative Analyses of Acthar Gel Indicate that It Does Not Contain Corticotropin

To investigate the content and character of the active ingredient in Acthar Gel, [*Lab Name*] [42] developed robust analytical test methods and performed cGMP-grade testing on

[42] [*Lab Name*] specializes in advanced biopharmaceutical testing for the purposes of substance characterization. It operates according to FDA current Good Manufacturing Practice regulations and the validation was done in accordance with the International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use ("ICH").

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[*Lab Name*] 's test methods, results, and analyses are referenced herein and attached as Exhibits. The results of [*Lab Name*] 's testing demonstrate that: **(1) Acthar Gel contains deamidated corticotropin; (2) the deamidated corticotropin is present in Acthar Gel at a concentration of approximately .21 mg/mL or 16.8 IU/mL; and (3) Acthar Gel does not contain a detectable peak corresponding to the non-deamidated corticotropin. These findings raise concerns as to the active peptide in the drug product, including the actual corticotropin concentration in Acthar Gel in view of the 80 IU / mL label claim of the drug product, and the potency required by the United States Pharmacopeia (USP) (80.0% - 125.0% label claim) for Acthar Gel.**

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Deamidation can result in changes in protein function, and this has been shown specifically for corticotropin. Graf et al. deamidated porcine corticotropin in 0.1 M ammonia solution at 37^0 C for six hours, then assayed the corticosteroidogenetic potency of the deamidated peptide in the peripheral blood of laboratory rats after intravenous administration. Graf et al. found a considerable decrease in the corticosteroidogenetic potency of corticotropin following deamidation, as shown in the below comparison of corticotropin and deamidated corticotropin (referenced as "ACTH" and "DACTH," respectively, in Table 1, below) and the researchers postulated that deamidation was responsible for the decreased biological activity.

Table 1. The biological activity of ACTH before and after deamidation

Peptide		IU/mg
ACTH	(a)	74.8 +/- 4.8
ACTH	(b)	91.2 +/- 8.5
DACTH	(a)	32.9 +/- 2.4
DACTH	(b)	49.0 +/- 2.7

Subsequently, Ekman et al. isolated and measured the biological activity of corticotrophic variants of porcine corticotropin. Ekman et al. determined that the fragment "F2" fit corticotropin 7-38, giving the expect amino acid structure up to residue 24, and demonstrating deamidation at the Asn-Gly structure at position 25-27. This fragment showed reduced corticotrophic activity, as compared to the intact porcine corticotropin 1-39 peptide, as measured by C_{50}, the molar concentration giving 50% of the maximal response, in isolated rat adrenal cells.

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In sum, therefore, [*Lab Name*]'s qualitative and quantitative analyses have demonstrated that:

(1) Acthar Gel contains deamidated corticotropin;

(2) Deamidated corticotropin is present in Acthar Gel at a concentration of approximately .21 mg/mL

(3) Acthar Gel does not contain a detectable peak corresponding to non-deamidated corticotropin

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3. Health Risks

[*Lab Name*] has shown that Acthar Gel contains deamidated corticotropin, and the tested Acthar Gel samples contain approximately .21 mg/mL deamidated corticotropin and fail to demonstrate a peak diagnostic for the presence of non-deamidated corticotropin (the only approved active ingredient in Acthar Gel). The reduction in potency of deamidated porcine corticotropin has been well-established. Moreover, Voigt et al. demonstrated that variants of porcine corticotropin are less active than the full 39-amino acid peptide in bioassays.

[*Lab Name*]'s findings thus raise concerns regarding the potency of Acthar Gel, changes in the physiochemical and functional properties of the active ingredient in Acthar Gel, changes in the stability of the active ingredient in the drug product, and immunogenicity issues that FDA may not have considered given Acthar Gel's unique regulatory approval pathway.

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4. Statutory and Regulatory Violations

The research data described above suggests several significant violations of the Federal Food Drug and Cosmetic Act (the "FDCA" or the "Act") that would merit a robust regulatory response.

(a.) Questcor's Acthar Gel Product, as Marketed, Is an Unapproved New Drug

…

FDA's approval was specific to repository corticotropin injection as that substance is defined by the official USP monograph. The product that Questcor is manufacturing and marketing today, however, does not conform to the compendia definition, and thus it is not what FDA approved based on a finding of safety and efficacy.

To our knowledge, Questcor has not presented clinical evidence that deamidated corticotropin is safe or effective for the treatment of infantile spasms, nor are we aware of any general recognition of such safety or efficacy. Curiously, Questcor itself has acknowledged the presence of numerous other ingredients, which it claims have therapeutic properties, but it has not identified these ingredients, and we are not aware of any evidence supporting such claims**.**

In the absence of clinical evidence supporting an approved new drug application for deamidated corticotropin or the other purported components in Acthar Gel, it is an unapproved new drug.

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(b.) Questcor's Acthar Gel Product Is Adulterated

Drugs manufactured in facilities or under conditions that do not comply with current Good Manufacturing Practice ("cGMP") regulations are adulterated within the meaning of the Act.

The cGMP regulations require manufacturers to ensure that products they make conform to specification. For example, manufacturers must have in place production and control procedures designed to assure that the drug products they produce have the identity, strength, quality and purity they are represented to possess. They must have in-process control procedures to monitor the output and to validate the performance of those manufacturing processes that are responsible for causing variability in the characteristics of in-process material and the drug product. They must test in-process material for the identity, strength, quality and purity as appropriate. They must test each batch of drug product to ensure conformance to final specifications, including the identity and strength of each active ingredient, and they must reject batches that do not conform. They also must maintain reserve samples of each distributed batch of product and conduct stability testing to ensure their products do not degrade and continue to conform to specifications within the designated expiration dates under appropriate storage conditions.

…

[*Lab Name*]'s test results described above suggest that Questcor's manufacturing facilities are not operating in a good state of control, and that perhaps due to failure to comply with one or more of the cGMP regulations, it is allowing product that does not comply with specifications to be released to the market. Such product is adulterated within the meaning of that likely will not deliver the desired therapeutic benefit.

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(c.) Questcor's Acthar Gel Product Is Misbranded

…

The official USP monograph identifies repository corticotropin injection as "corticotropin in a solution of partially hydrolyzed gelatin. Its potency is not less than 80.0 percent and not more than 125.0 percent of the potency stated on the label in USP Corticotropin Units. The label for Acthar Gel says it contains "80 USP units per mL". Thus, to comply with the USp monograph – and be properly labeled as repository corticotropin injection – it must contain between 64 (80%) and 100 (125%) units of corticotropin.

In fact, Acthar Gel contains far less than 64 units of corticotropin; indeed it appears not to contain any corticotropin at all. It is not, therefore, repository corticotropin injection, as defined by the USP and as represented in its labeling, and thus it is misbranded in violation of Section 502 of the Act.

This is a significant violation. As explained above, it is well recognized in current literature that when corticotropin is deamidated, it loses much of its potency. It is therefore hard to say what, if any, therapeutic value Acthar Gel can have, since it seems to contain so little of the active ingredient that was the basis for the FDA's approval.

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FDA's drug approval process is predicated on the principle that sponsors of the new drug applications will come forth with specific, identifiable, active pharmaceutical ingredients, formulated to particular specifications, and demonstrate their safety and efficacy for treating particular indicated diseases.

The available evidence suggests that Questcor has turned that process on its head with Acthar Gel. The product it seems to be manufacturing is not the product it described in its application for approval for a new indication, nor is it the product the FDA originally approved in 1952. And Questcor's Acthar Gel is not the drug product in studies that purport to show its therapeutic benefit for infantile spasms, an indication that Questcor has estimated to represent less than 10% of total sales.

Drug manufacturers have an obligation to ensure that the drugs they make conform to the specifications established in their applications. If their products do not conform, neither the FDA, treating physicians, nor patients can know that marketed drugs are safe and will deliver the desired benefit. We are concerned, based on the evidence reported here, that Questcor is not meeting that obligation with Acthar Gel, and therefore strenuously urge the Agency to investigate.

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If you have any questions about [*Lab Name*] 's findings, or the test methods it used, we would be more than happy to answer them. Please do not hesitate to contact me with any such questions and we will endeavor to get you answers immediately.

Thank you for your time and attention to this problem.

Sincerely,
[NAME OF ATTORNEY]

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Lab Reports Citron has Reviewed in Preparation of this Report

Lab Doc 0002: Pages: 62
Date: July 2013
Method: LC/MS/MS
Separate Spectroscopic Measurement Graphics: 172
R&D Grade Testing of a 4.5 kDa Peptide rug Product by "LC/MS/MS"

Conclusion: **This report describes the R&D grade testing of four vials of drug product with assigned CSTNs [REDACTED] …. These samples were analyzed by LC-MS/MS in triplicate according to the method described in [method description document], where they were compared to Sigma ACTH controls (deamidated and non-deamidated), which were prepared in the same way as the test samples. The resulting intact mass measurements and MS/MS fragment ion spectra are similar to those obtained from the deamidated Sigma ACTH control and indicate that all test sample vials (opened and unopened) contain iso-aspartate or aspartate at position 25 and glutamate at position 30.**

(Lab Document 0002)

Lab Doc 0003: Pages: 87
Date: Oct. 2013
Method: LC/MS/MS
Separate Spectroscopic Measurement Graphics: 258
Comparison of Acthar against controls (known deamidated and non-deamidated ACTH)

Summary: **This report describes the cGMP testing of four vials of drug product with assigned CSTNs [REDACTED]. These samples were analyzed by LC-MS/MS in triplicate according to the method described in [method description document], where they were compared to Sigma ACTH controls (deamidated and non-deamidated), which were prepared in the same way as the test samples. The resulting intact mass measurements and MS/MS fragment ion spectra are similar to those obtained from the deamidated Sigma ACTH control. Detectable levels of ACTH (with Asn at position 25) were not observed, which suggests that the majority of ACTH is degraded in all the samples analyzed.**

(Lab Document 0003)

Lab Document 0004: Pages: 47
Date: October – November 2013
Method: LC/MS
Separate Spectroscopic Measurement Graphics: 162
Method Development and R&D Grade Testing for Quantitation of a 4.5 kDa Drug Product by LC/MS

Summary: **This report describes the Method Development and R&D grade testing to quantify the amount of a 4.5 kDa peptide drug product by LC/MS. Based on earlier experiments documented in [LABORATORY], where only deamidated ACTH could be detected, an LC/MS method was developed for the quantification of deamidated ACTH from a gelatin matrix, using a standard curve generated from a standard synthetic ACTH (Asn) peptide and a heavy isotopically-labeled ACTH (Asp) variant peptide as an internal standard. The isotope-labeled heavy standard was employed to correct for any peptide losses that occur during sample preparation; it contains an aspartate instead of an asparagine and therefore has the same structural sequence as deamidated ACTH. …**

(Lab Document 0004)

Lab Document 0005: Pages: 34
Date: October – November 2013
Separate Spectroscopic Measurement Graphics: 63
Method Development for the Analysis of a 4.5kDa Peptide Drug Product by LC/MS/MS

Summary: **A robust method has been developed to analyze the client's drug product (ACTH formulated in 16% gelatin) by LC/MS/MS. Several methods were evaluated in order to provide the best deformulation condition that enrich ACTH from the test sample and remove the gelatin proteins prior to analysis. Precipitation with fifty percent acetone provided the best recovery of ACTH from the gelatin matrix, and the HPLC autosampler was modified to inject large (300uL) diluted aqueous solution of this solvent containing ACTH. In addition, chromatographic separation of structural isomers of ACTH in the sample was achieved using a Poroshell 120 C18 column (Agilent). This also provided robust and reproducible chromatography that can be compared directly to the traces observed for ACTH and deamidated ACTH and the intact mass data and fragment ion spectra produced by LC/MS/MS corroborated this assignment.**
…

(Lab Document 0005)

Lab document 0006: Pages 19
Separate Spectroscopic Measurement Graphics: 12
Date August 22, 2013
Analysis of Drug Product by LC/MS/MS

Lab document 0007: Pages 16
Date November 18, 2013
Quantitation of Drug product by LC/MS

Lab document 0008: Pages 11
Date November 18, 2013
Quantitation of Drug Product by LC/MS (Study Specifications)

Notes on the Lab Testing:

- **Principal findings: little to no ACTH**
- **Measurable amounts of deamidated ACTH, but far below labeling specification for the ACTH concentration**
- **Synthetic ACTH not detected**
- **Results from two independent laboratories**
- **Tested product from two separate Acthar batches, consistent results from both samples**
- **Testing methodology compared against known pure ACTH and deamidated ACTH control arms**
- **Per good laboratory practice, the method of testing and measurement was determined, documented and signed off before the tests were run**
- **State of the art Liquid Chromatographic / Mass Spectrographic analysis test equipment employed**
- **Over 650 detailed graphical readouts from analysis equipment included in the lab report documentation**

Does Any of This Makes Sense? Of Course it Does

To add more context to this story, just earlier this month Questcor slipstreamed into a "management team" PR the abrupt mention that that their head of manufacturing and CTO, David Medeiros, an 11 year veteran, is being transitioned to a "non- executive advisory role". Medeiros was the most knowledgeable person about the complete process of manufacturing Acthar at Questcor. There is no succession plan offered other than Questcor claiming it is initiating a search for a replacement. Hmmmm…
http://ir.questcor.com/releasedetail.cfm?ReleaseID=823358

Meanwhile, Questcor has never completed a clinical trial for any Acthar indication, and even the trials that have been published on Infantile Spasms have come back as inconclusive over the years.
https://blogs.law.harvard.edu/billofhealth/2013/01/08/at-28000-a-dose-how-effective-is-acthar/
and
https://blogs.law.harvard.edu/billofhealth/2013/01/07/regulatory-concepts-in-the-news-part-i-fda-efficacy-standards-for-old-drugs/#more-3895

Questcor abruptly bought their supply manufacturer and its facility – Now it Makes Sense

When, out of nowhere, Questcor began to make claims about unknown lesser ingredients, what they possibly were really doing was positioning to cover up the mislabeling of the active ingredient.

Lastly, the arrogant and cryptic answer given by CEO Bailey to the New York Times can now be seen within its fuller context. He has claimed repeatedly that Acthar is a biologic, its manufacturing is a trade secret, and its composition is "undisclosed". Here's where he compared it, on record, to a "secret sauce" formula such as the one for Coca-Cola. He gave this quote to the New York Times.

" ONE big uncertainty hanging over Questcor is competition. As an old drug without patent protection, Acthar would seem to be a sitting duck for generic rivals. And other versions of ACTH have been sold in the past.

Yet Questcor is now arguing that its studies show that Acthar, despite the "highly purified" in its name, actually contains other substances from the pig pituitary glands that account for some of its effectiveness. The company does not intend to say what those other ingredients are, thus making it extremely hard for a generic company to copy Acthar.

"Coca-Cola is not going to tell you what Coke contains, either," Mr. Bailey says.

http://www.nytimes.com/2012/12/30/business/questcor-finds-profit-for-acthar-drug-at-28000-a-vial.html?pagewanted=1

The US Food and Drug Act was incepted **precisely** to bring to an end to this type of "snake oil" claim – unproven medicinal claims for products with "secret ingredients" that have not proved safety and efficacy.

Questcor's drug comes to market under a grandfathered label for purified ACTH, not an unknown compound-ingredient biologic. So its claim with regard to pending competition is that there are "some other ingredients other than ACTH, which give it unique efficacy" – although there is no modern clinical evidence of that efficacy.

What **is** known about these substances – which we now know to be deamidated ACTH, is that there are findings that observe **decreased** biological activity compared to ACTH.

These findings on the real content of Acthar were formally reported to the FDA over two months ago. The submitter has top credentials; he is not some hedge fund guy complaining about a pharma company for his own self interest. He is a former FDA legal official, with specific experience and enforcement authority in the area of seizure, injunction, and civil penalties.

Questcor's Respsonse

We have no doubt that Questcor is going to respond to this report by stating their relationship with CSL Behring, who they retain to test the drug for toxicity and potency. Do they supply CSL Behring with true random sample of production batches, or is the relationship more casual for the potency testing?

While we do not know this answer, we do know that over the years Questcor has become experts at exploiting loopholes. Most importantly, we have had recent conversations with former top executives at Questcor who have verified that it is not easy to deamidate Acthar – the results we share today are very unlikely to be "an accident" or a lab anomaly. The lab results we report cannot be easily dismissed with excuses.

Of all the conversations we have had with former employees and executives at Questcor, one observation stands consistent. Questcor keeps its manufacturing a complete secret. It does not allow people to visit the plant and keeps that plant firewalled from the rest of its corporate structure.

It is Citron's belief that these findings, and the inquiry on which they are based, is highly legitimate and credible. Obviously, Questcor is a battleground stock, and the analysts have already made up their minds. But these questions change the landscape for Questcor investors.

Questions we can't answer today … but they're Vitally Important to the Future of Questcor.

- How long has Questcor known that there's little or no ACTH content in shipped Acthar? Is it conceivable that Citron tested a "bad batch"?
- Has Questcor been contacted by the FDA over the past month and been required to surrender sample vials and records (that would explain corporate departures and outsized insider sales)
- What is Questcor's exposure to patient lawsuits?
- Do they manufacture some Acthar that has genuine ACTH content, and other batches that do not? If so, how do they decide on which product from which batch to ship to whom? Do the infantile spasms prescriptions get the good product? How about their own clinical trials? How about their audit samples?

- Does this finding expose Questcor to a cease-and-desist from FDA on current sales? Will they be required to submit new clinical trials before being allowed to resume sales?
- Does this corporate act – shipping mislabeled product -- lead to a regulatory challenge to their purchase of Synacthen rights?
- How can any going-private transaction or buyout rumor proceed with the regulatory threat to Questcor losing rights to its only source of revenue?
- Why does Questcor not have a system in place that can present adverse events to the FDA? Unlike other pharmaceutical companies it appears that Questcor is delinquent in its Adverse Event disclosure.

We assume most of these questions will be answered by the FDA or in a court of law.

Why it is Especially Important for the FDA to Take Action Now

Investors and regulators must understand that Acthar is not a drug that is being prescribed by doctors nationwide through ordinary marketing channels based upon its efficacy. Rather, for MS and to some extent for the other major indications that comprise the bulk of its revenues, the demand for Acthar is patient driven. It is a direct result of aggressive marketing efforts by Questcor.

Questcor runs seminars for MS patients with the inducement of "free dinner" … during which a presenter from their "Speaker's Bureau" pitches them on the idea of getting their physician to prescribe Acthar for exacerbations of their MS.

Below is a table summarizing Questcor's upcoming events over the next 60 days that shows the scope of their marketing machine. This is a drug that is being marketed directly to patients in need by doctors and other medical professionals that are being funded by Questcor.

Let's be clear: We're talking about a **huge** number of events. Here's how the calendar looked in late January 2014:

Report Date	Upcoming events	January 2014	February 2014	March 2014	April 2014	May 2014
02/19/14	211	88	156	110	19	5
02/12/14	205	88	153	87	13	3
02/05/14	200	88	147	54	8	2
01/29/14	179	88	137	36	5	1
01/22/14	162	88	114	23	2	
01/15/14	163	88	90	14	2	
01/08/14	152	86	61	11	2	
01/02/14	149	82	56	9	2	
12/26/13	149	82	56	9	2	
12/18/13	132	76	38	7	1	
12/11/13	132	60	19	5	1	
12/04/13	157	39	14	5		

Questcor has an entire team booking and managing these events. They are staging over 200 per week! That's 10,000 free-dinner events per year all to pitch a drug that is mislabeled and whole active ingredients are unknown to the FDA and the scientific community.

Incredulous? Here is a live view of their event calendar:
http://www.msviews.org/msviewsandnews4/index.php/2012-05-28-00-15-54/questcor-patient-program-calendar

Add to this perspective that because of the influence of this marketing campaign, patients themselves are being placed at the center of treatment decisions with regard to a drug that, needless to say, they have less than the full spectrum of information available to them on which to make health care decisions that are best for themselves and their own situation.

Conclusion

This report is not just another data point. It is **the** data point. It is the only data point.

Therefore, in this report Citron is not going to address the multiple investigations into Questcor, massive insider selling, or details of the aggressive marketing tactics that we have uncovered. The gravity of this report cannot be overstated. This information must be evaluated on its own merits by each investor.

With regard to this single point, all the bullish investment theses: new indications, insurance reimbursement, and increasing revenue curves are entirely irrelevant. There is only one question: **What is in the vials of Acthar Gel?**

Beyond a stock story, this is a story of sick and desperately ill people receiving treatment that is not accurately dosed, and whose active ingredients do not match what is listed on the label as filed with the FDA, and are not clinically proven by modern standards. Everything else about this company is irrelevant. Insurance, charity co -pays, free drugs, indications, and marketing all mean NOTHING if the product being sold is in violation of the Food and Drug Act and not distributed to the public consistent with its FDA approval.

At the turn of the year, respected biotech commentator Adam Feuerstein, listed his annual predictions for 2014. He opined that ongoing Federal investigations of **Questcor Pharmaceuticals**, and specifically its marketing practices for its flagship product Acthar Gel, would culminate in civil and criminal indictments against the company, forcing CEO Don Bailey to resign. There was reason enough for that prediction, but we bet in his wildest dreams Adam never thought his prophecy would come true for **this** simple reason:

What is Really in HP Acthar Gel?

- **As always, cautious investing to all.**

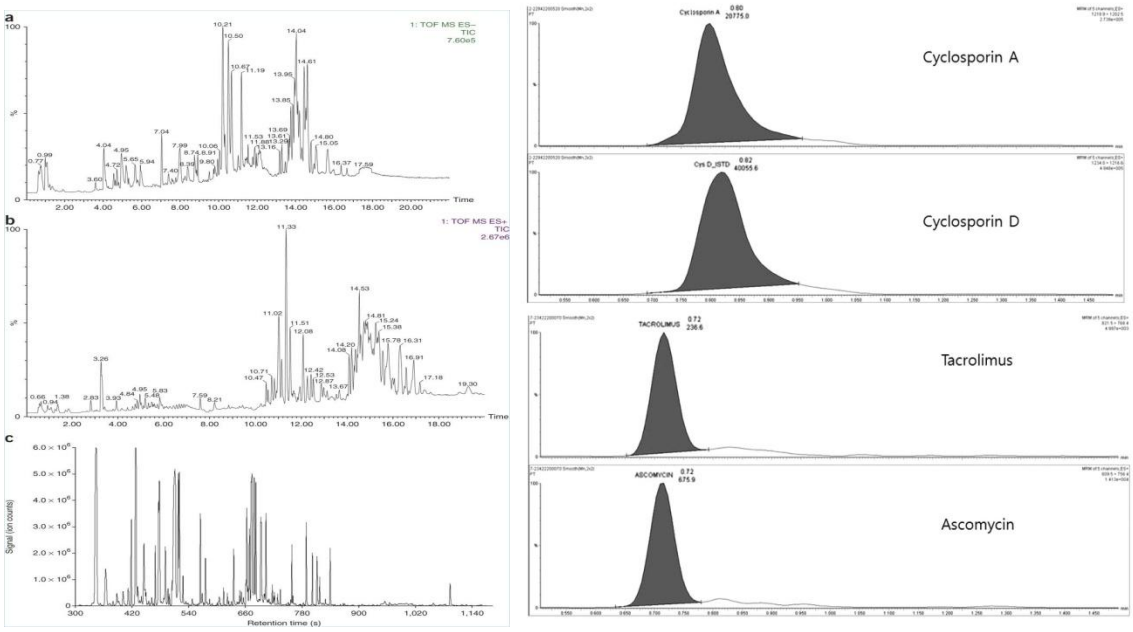
… But just one more thing …

The Data—Explanatory Appendix

We're sure the data in the studies undertaken by these research labs will be debated, disputed, and denied. Citron is not scientifically qualified to preside over this debate. We will have to confine ourselves to what we've seen.

First of all, these studies come out of two highly-credentialed, highly respected research laboratories with specific expertise in product characterization in the context of cGMP compliance. The main technology used for this analysis is called LC-MS (and a closely related technology LC-MS/MS), combining the biochemists main tools of Liquid Chromatography and Mass Spectrometry.

The scientific findings they produced while studying Acthar are replete with the graphical outputs of the high-technology machines that do this type of work. There are in fact over 650 separate measurement graphics in the reports, nearly all of which are the form of these generic samples:



In order to protect the privacy and integrity of the lab performing the studies, which are ongoing, we haven't published any of the actual study graphs in this report. However, if you're curious enough to have read this far, you are entitled to know the depth to which this work has gone and is continuing to go.

This study is expensive, thorough and ongoing. It is professional enough in nature to be suitable to present to the FDA. And it is as unambiguous in its findings as it is thorough in its scientific approach. -- *Editor*