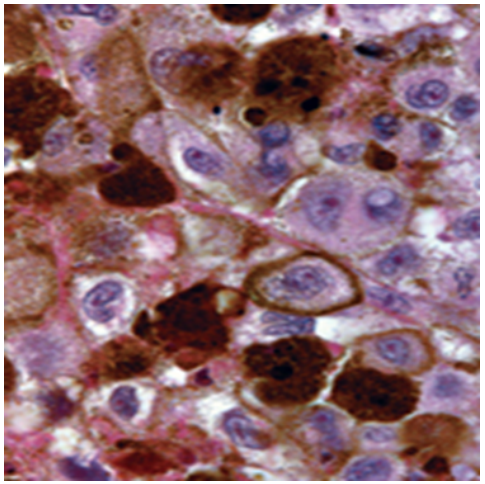

CSCs: The Demon Seed

Henry McCusker

Scimitar Equity

Take a moment…

- Half of us in this room will get cancer, one-third will die from it.
- Tens of thousands of compounds go through the lab…
- But few succeed.

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About Scimitar Equity

- Scimitar Equity is an independent equity research firm focused on providing value-added intelligence and insightful investment conclusions on emerging companies demonstrating real progress toward their vision while meeting quarterly expectation.

- Scimitar Equity's Regenerative Medicine Investors aggregates, curates and creates bottom-line content weeding of sector news to provide a customized, vetted selection of relevant and high-impact synthesis. Since 2001, as an independent advisory, online news and digital media publisher; our mission is to provide readers with exclusive, in-depth coverage of cell therapy companies, technology, and investing funds. Our vision is to provide time sensitive intelligence and metrics to define the ever changing economics of regenerative medicine.

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Henry's Perspective

Publicly Traded Companies
Targeting Cancer Stem Cells

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Why Stem Cells

- Stem cells have recently generated more public and professional interest than *any other topic in biology*
- Why? Understanding their unique properties may provide deep insight into the building blocks of cancer as well as the path towards treatment
- Investors understand cancer therapeutics and pipelines, but don't understand the applicability of stem cell therapy and regenerative medicine
- That's why many companies in the CSC universe differentiate their focus **NOT** to be included in the RegMed sector.

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Why Focus on Cancer Stem Cells

- Current treatment standards for cancer use chemotherapy and radiation targeted at tumor bulk
- Yet what often remains are CSCs, the underlying cause of tumor recurrence and metastasis
- So like the bad seed, CSCs are the clinician's nemesis as they are the central cause of cancer reappearance
- The mission – vanquish the demon CSC and destroy the cancer.

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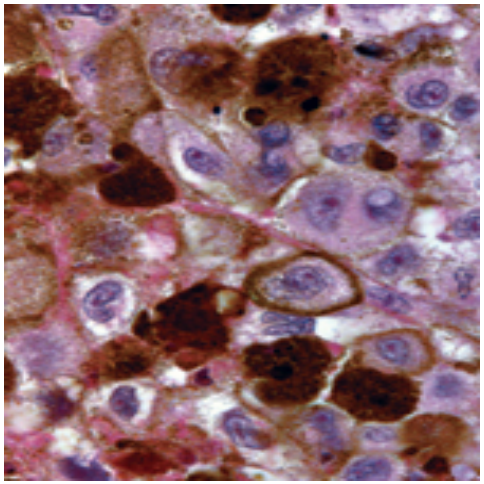

Cancer stem cells are defined as those cells within a tumor that can self-renew and drive tumorigenesis.

Rare cancer stem cells have been isolated from a number of human tumors, including haematopoietic, brain, colon and breast cancers.

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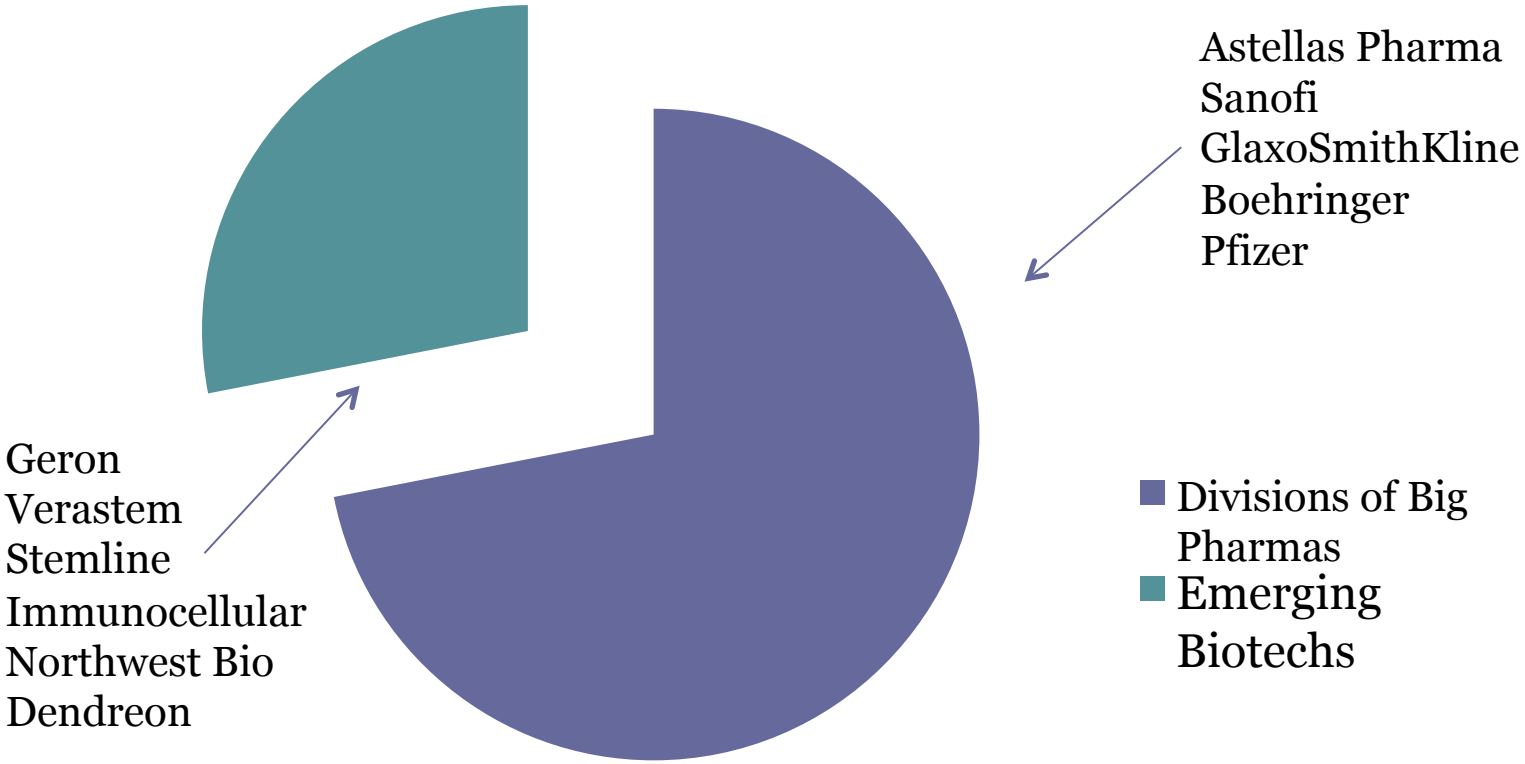
CSC Universe



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Henry's Criteria

- Market Cap
- Management
- Research – Separate Science Fact from Science Fiction
- Burn Rate

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Verastem Dollars and Cents

- Market Cap: $195.65 M
- Management: Good to excellent
- Current price: $9.19 (5/17/2013)
- 52 Week Range - $6.25 - $12.00
- Quarterly burn rate +/- $6.5 M (Q4 2012 – Q1 2013)

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Verastem Focus

- *Kill the signal, kill the stem cell, kill the cancer*
- Discover and Develop drugs to treat cancer by targeted killing of cancer stem cells (CSCs).
- Has identified a pipeline of small molecule compounds with the potential to target CSCs.
- Most advanced programs target Focal Adhesion Kinase (FAK) and PI3K/mTor **signaling pathways in highly invasive-highly metastatic cancers**
  - Verastem small molecule FAK and PI3K/mtor inhibitors preferentially target cancer stem cells and reduce tumor-initiating capability.
- I'll defer to Jonathan Pachter, Chief Scientific Officer of Verastem to say a little more on the science and the clinical trial plan and any results

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My View

VSTM just "trudges" along in the preliminary stages of clinical development with twists and turns but nothing that upsets the apple cart - but great cash position!

The combination trial of VS-6063 plus paclitaxel for ovarian cancer is open and enrolling patients at all sites. In addition, VSTM has met with the regulatory agencies in the US and UK and are on track to initiate midyear the potentially pivotal trial of VS-6063 in mesothelioma. 2013 milestones include the following: Complete the dose finding portion of the P1/1b trial of VS-6063 plus paclitaxel in ovarian cancer; Initiate P1 clinical development of VS-4718 H1 2013 and Initiate P1 clinical development of VS-5584 H2 2013. A ...HOLD as I don't see any great expectation in a Q or 2!

Scimitar Equity Research Report May, 2013

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Stemline Dollars and Cents

- Market Cap- $50.75 M (COB 5/17/2013)
  - 5/16/13 – Prices $60M public offering of common stock
- Management: I have a few questions…
- Current price: $15.00 (5/17/2013)
- 52-week range $10.33 - $17.00
- Quarterly burn rate: $2.26 M

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Stemline Focus

- Developing novel oncology therapeutics that target cancer stem cells (CSCs) as well as the tumor bulk. Among Stemline's drug candidates are SL-401 and SL-701,
- **Both have demonstrated clinical activity including durable complete responses (CRs) and an overall survival (OS) benefit versus historical controls in PI/II studies.**
- 1/7/2013 - a second heavily pre-treated patient with a drug-refractory and recurrent blastic plasmacytoid dendritic cell neoplasm (BPDCN) **achieved a complete response (CR)** following treatment with a single cycle of Stemline's lead therapeutic, SL-401. To date, 2 of 3 patients with drug-refractory BPDCN experienced CRs following treatment with SL-401.

Stemline My View

Investor demand for shares of Stemline Therapeutics (NASDAQ: STML) seems to be picking up tremendously, as the stock rallied 13.3% higher yesterday to reach a new high of $16.49 per share in yesterday's trading (May 1st, 2013). This represents a 65% premium over its IPO price of $10.00 per share, and shows that there has been significant investment interest from the market since January 2013. Through the public offering, Stemline collected $38.15 M in proceeds. Based on the statements of CEO Dr. Ivan Bergstein, this should provide the company with the funds needed to meet the next key milestones for its SL-401 and SL-701 development programs.

STML closed at $14.96 on 5/3/13 and has 3.44 M shares outstanding and a float of $2.09 M. So, why not buy … not at this price which will suffer a little by dilution. STML is already DOWN -$0.62 or -4.14% to $14.34 – look for it to even out at $14.00. HOLD!

Scimitar Equity Research Report May, 2013

Scimitar Equity

geron Dollars and Cents

- Current market cap - $146.08 M (COB 5/17/2013)
- Management – New, but has made tough choices
- Current price: $1.12 (5/17/2013)
- 52-week range $0.91 – $2.99
- Quarterly burn rate: $12.8M

Scimitar Equity

geron Focus

- Development of a first-in-class telomerase inhibitor, imetelstat, in hematologic myeloid malignancies
- Imetelstat inhibits the activity of telomerase, an enzyme present in most types of cancer that enables tumor cells to replicate indefinitely.
- GERN's potential anti-cancer strategy is to stop or slow down the growth and spread of tumors by inhibiting telomerase activity.
- Investigator-sponsored study at Mayo Clinic
  - an open-label trial in intermediate or high-risk patients with primary or secondary MF.
  - primary endpoint is overall response rate, defined by the proportion of patients who are classified as responders having achieved either a clinical improvement, partial remission, or complete remission
  - Initial first cohort of 11 patients enrolled
  - Prespecified criteria in the clinical protocol of at least two responders was met – will enable expanding enrollment

geron My View

GERN after the restructuring has a net loss of $11.9 M or $0.09 per share wider than the Consensus Estimate of a loss of 8 cents but narrower than the year-ago loss of 15 cents. Q1/13 revenues of $765 K were well below the year-ago revenues of $1.3 M. A significant decline in expenses led to the narrower loss. Revenues consisted entirely of royalties and license fees. Total operating expenses declined 36.6% to $12.8 M. R&D expenses declined 47% to $8 M due to reduced personnel-related costs and lower costs related to the manufacturing of imetelstat and GRN1005 as well as lower costs resulting from the… winding-down of the imetelstat (metastatic breast cancer and advanced non-small cell lung cancer) and GRN1005 (brain metastases) studies. G&A expenses declined 5.9% to $4.8 M, mainly due to lower personnel-related costs.

GERN now intends to focus on developing imetelstat for hematologic myeloid malignancies and does not plan to study the candidate in non-small cell lung cancer with short telomeres or essential thrombocythemia (ET).GERN discontinued its discovery research and companion diagnostics programs and will shut down its research laboratory facility. GERN's enrollment of the 1st cohort of patients in an investigator-sponsored study on imetelstat at Mayo Clinic for myelofibrosis (MFGLQ) was completed at the end of March. With pre-specified criteria in the clinical protocol being met, expanded enrollment should continue. Other investigator-sponsored trials this year could be on hematologic myeloid indications like acute myelogenous leukemia and myelodysplastic syndromes. HOLD

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ImmunoCellular
Immunocellular Therapeutics Ltd.

Dollars and Cents

- Current market cap - $134.98 M (COB 5/17/2013)
- Management – New CEO, wanting for metrics
- Current price: $2.56 (5/17/2013)
- 52-week range $1.51 - $4.00
- Quarterly burn rate: $2.35 M

Scimitar Equity

ImmunoCellular
Immunocellular Therapeutics Ltd.

Focus

- Developing novel immune-based products to treat cancer.
- Active Immunotherapies target regular tumor cells and cancer stem cells
- Most advanced clinical programs are in glioblastoma multiforme (GBM)

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ImmunoCellular
Immunocellular Therapeutics Ltd.

My View

Good cash, better management and based on "earlier" trial data (although a small patient population) – IMUC has “good” prospects for clinical success. Interim analysis of the ongoing ICT-107 P2 clinical trial and of the interim analysis by the data monitoring committee is anticipated in Q2/13 and the first top-line results are is expected to be available by the end of this year. IMUC completed enrollment for the P2 trial of ICT-107 in patients with newly diagnosed glioblastoma, and that trial continues to progress well. 2 INDs, for ICT-121 and ICT-140 are currently active, with plans underway to begin clinical trials for both programs. IMUC brought a 2nd manufacturing site on-line for ICT-107, advancing its ability to meet the needs of further clinical testing. The controversy is over their claim that they could possible treat 75% of patients with “glio”. The actual treated patients in the trial would indicate that only about 50% would make the cut. The problem is IMUC has never given an adequate explanation of why they still think it could be 75%. HOLD.

Scimitar Equity Research Report May, 2013

Scimitar
Equity

NORTHWEST BIOTHERAPEUTICS

Dollars and Cents

- Current market cap - $111.69 M (COB 5/17/2013)
- Management – more than a few questions
- Current price: $3.71 (5/17/2013)
- 52-week range $2.98 - $20.00
- Quarterly burn rate: $6.5 M

Scimitar Equity

NORTHWEST BIOTHERAPEUTICS

Focus

- Discovering, developing and commercializing immunotherapy products that generate and enhance immune system responses to treat cancer.
- NWBO's platform technology, DCVax®, uses a patient's own dendritic cells, the starter engine of the immune system. The dendritic cells are extracted from the body, loaded with tumor biomarkers or "antigens", thereby creating a personalized therapeutic vaccine.
- Injection of these cells back into the patient initiates a potent immune response against cancer cells, resulting in delayed time to progression and prolonged survival.

Scimitar Equity

NORTHWEST BIOTHERAPEUTICS

My View

NWBO is an enigma to many – as many pluses as there are negatives. They have 30.1 M shares outstanding with a float of 14.8 M shares trading in a 52 week trading range of $2.98 - $20.00 with erratic volumes. CEO Linda Powers also has had her list of detractors. Extrapolating from Larry Smith (on Stocks) … another friend and former H&Q alumni …"I am encouraged by the progress made by the Northwest Biotherapeutics (NWBO) over the past year and maintain my Buy recommendation. I <Larry>first began coverage in July of 2012. At that time it was a bulletin board company with $430,000 of cash on its balance sheet, $31 M of debt and $1 M of assets. Not surprisingly, most investors regarded the Company as being on a path to going out of business. Since the financial crisis of 2008, NWBO had survived through doing small offerings nearly on a quarterly basis that resulted in the issuance of a large number of shares and warrants. If the balance sheet was not enough to give pause, Wall Street has had mostly bad experiences with companies involved in cancer vaccine developments and there was almost no institutional interest. Characterizing the situation as grim might be an understatement.

Northwest believes that the key to efficacy is identifying at what point the immature dendritic cells are most capable of both taking up antigens and displaying them. The cells in DC Vax Direct are partially mature dendritic cells somewhere in the maturation stage between monocytes and mature dendritic cells. NWBO believes that prior development efforts used cells that were either too immature or too mature. It believes that it has identified a dendritic cell maturation stage that will work. This requires both precision and control of the manufacturing process in order to consistently replicate this batch after batch. DC Vax Direct will begin a multi-center P1/2 trial in Q2/13.

Dendreon
Targeting Cancer, Transforming Lives™

Dollars and Cents

- Current market cap - $610.44 M (COB 5/17/2013)
- Management – a lot of changes to re-guide the ship with a compromised hull
- Current price: $4.01 (5/17/2013)
- 52-week range $3.69 - $7.84
- Quarterly burn rate: $126 M

Scimitar Equity

Dendreon
Targeting Cancer, Transforming Lives™

Focus

- Discovery, development, and commercialization of novel therapeutics to enhance cancer treatment options for patients.
- Product portfolio includes active cellular immunotherapy and small molecule product candidates to treat a range of cancers.
- DNDN maker of PROVENGE, an autologous cellular immunotherapy for the treatment of asymptomatic or minimally symptomatic, metastatic, and castrate-resistant prostate cancer.
- Product candidates under development comprise DN24-02, an investigational cellular immunotherapy, which is in PII clinical trials for the treatment of patients with bladder, breast, ovarian, and other solid tumors expressing HER2/neu; and carbonic anhydrase 9, an antigen expressed in renal cell carcinoma that is in preclinical development.

Scimitar Equity

My View

DNDN is DOWN -$0.07 to $3.96 post-earnings from a “thrashing”! Closed at $4.03 on 5/9/13 – let it free fall … but… could a potential $3.50 – $3.65 share price represent - a serious buying potential – I’d take that bet twice but with also a therapy pricing reduction – which I believe is coming!

Pricing the therapy is still …the “electronic” collar on this dog’s neck! Love the cash position but hate the campaign to bury the company hoping the direct-to-consumer campaign which began in March to educating potential patients in the mCRPC market employing enhanced sales messaging responds better with customers … for the quarter for Dendreon was absolutely dreadful. Revenues completely fell apart, falling well below even the most bearish of analysts’ and whisper numbers. The company’s Q2 revenue guidance detailed another year-over-year drop. The company also burned through a substantial portion of its cash and investments pile. Xtandi is approved for patients who haven’t been helped by chemotherapy. Zytiga and Xtandi are pills, while Provenge is given in three infusions over the course of four weeks. Provenge is approved for men whose cancer has spread elsewhere in the body and has not responded to hormone therapy or radiation. It is designed to train a patient’s immune system to fight prostate cancer and a round of treatment costs $93,000. All the negativity will drive DOWN the stock

So what…

- CSCs therapies and their associated equities are in their infancy, what's often needed is a buy low and sit tight strategy – however the nature and vagaries of this traders market begets a whole new paradigm.

- Why? Sophisticated investors recognize that CSC's ultimate potential is still years in the future, but play more the peaks and valleys than invest for the long term. As the mini-sector matures and the hope that therapies in development become standard forms of treatment, it will then be time to reap rewards of reality-based clinical data.

- The jury is still out whether CSC companies will become hot takeout targets for Big Pharma. Pharma typically prefers companies with more tangible products and concrete clinical data. For Pharma to take something out, they need more data. Also, pharma is still getting used to the idea of cellular therapies.

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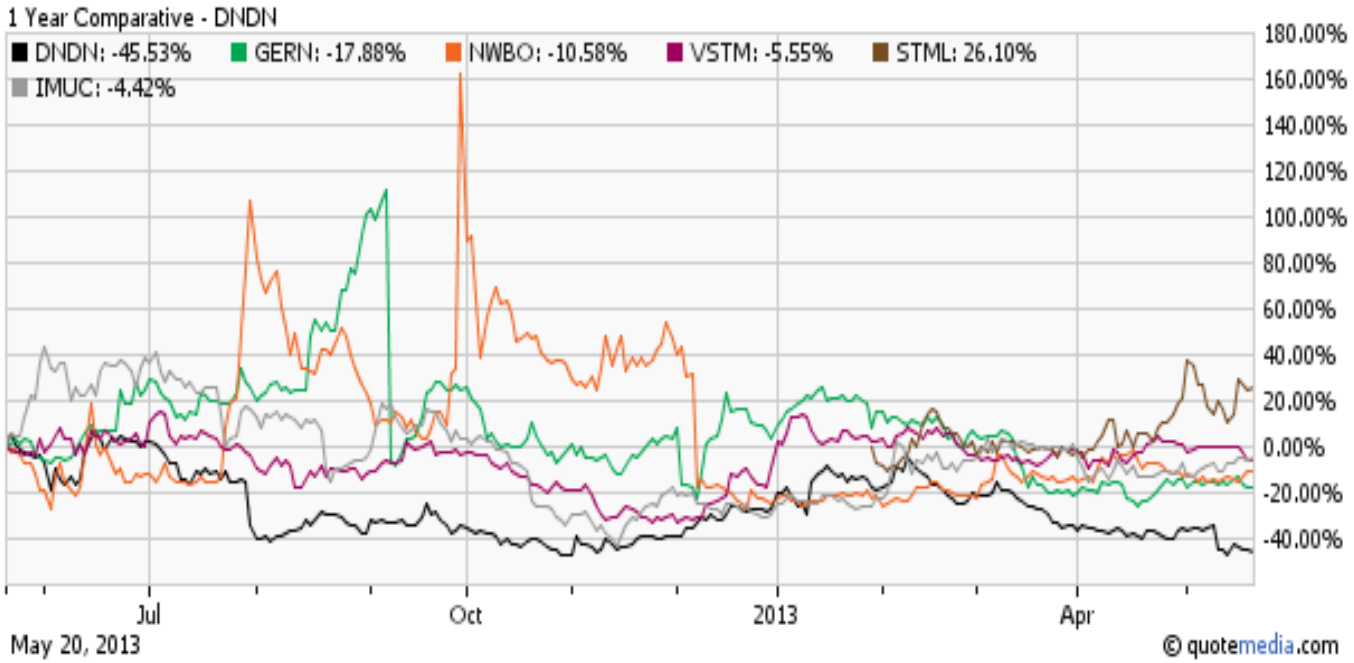

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Thank You!

SACHS ASSOCIATES

Annual CANCER BIO PARTNERING FORUM
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